



# *Impact of Payer Coverage and Reimbursement Policies on Off-Label Use of Anticancer Therapies*

**Final Report**

**Prepared by  
Covance Market Access Services Inc.  
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# *Table of Contents*

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■ Introduction and Methodology	Page 3
■ Key Findings	Page 9
■ Current Off-Label Use of Anticancer Therapies	Page 18
■ Coverage and Reimbursement of Off-Label Anticancer Drug Use	Page 32
■ Recent Medicare Policy Changes	Page 45
■ Conclusions and Recommendations	Page 47
□ Appendix A: Oncology Practice Survey Tool	
□ Appendix B: Oncology Practice Characteristics	
□ Appendix C: Background on Medicare's Coverage Policy for Off-Label Use of Anticancer Therapies	

# *Introduction and Methodology*

# *Study sponsors\* are interested in investigating the role of off-label prescribing and the impact of payer coverage policies.*

## **Research Questions**

- Approximately how many oncology patients (not visits) do practices see per month?
- How important is off-label use of anticancer therapies to the treatment of practices' cancer patients?
- Why is off-label use of anticancer therapies important to the treatment of practices' cancer patients?
- What sources of information do practices rely on to make off-label treatment decisions for anticancer therapies?
- How important are drug compendia and peer-reviewed medical literature to practices' use of off-label anticancer therapies?
- How, if at all, have practices' frequency of prescribing off-label use of anticancer therapies changed over the past five years?
- How often do local Medicare contractors' and private payers' coverage and reimbursement policies for off-label use of anticancer drugs cause practices to alter their clinical decisionmaking?
- How often do local Medicare contractors' and private payers' coverage and reimbursement policies restrict practices' off-label use of anticancer therapies?
- What sources do payers use to support coverage for off-label uses of anticancer drugs? What methods have Medicare contractors and private payers used to become more restrictive with coverage and reimbursement for off-label uses of anticancer drugs?
- How often do practices encounter coverage denials for off-label uses listed in drug compendia as "accepted" indications? Not listed in drug compendia but supported by peer-reviewed medical literature?
- In cases where payer coverage or reimbursement policies for particular off-label uses of an anticancer drug are restrictive, how do practices usually respond?

\* Association of Community Cancer Centers (ACCC), Biotechnology Industry Organization (BIO), and Pharmaceutical Research and Manufacturers of America (PhRMA).

Please refer to Appendix A for our complete oncology practice survey tool.

## *To better understand oncologist and practice manager perceptions of off-label use of anticancer therapies, Covance surveyed office-based oncology practices.*

- Covance used the Internet-based Inquisite™ survey software package to conduct the survey.
- Using ACCC's comprehensive member list, Covance sent survey invitations to nearly 3,500 office-based oncology practices.
- Oncology practices were given 3 weeks to complete the survey (June 3, 2008, to June 24, 2008).
- Respondents were not compensated for their participation in the survey.
- Covance received a total of 165 responses.
- This survey was designed as a follow up to a smaller, in-depth study of oncologists and oncology practice managers conducted by Covance in 2005, which identified important areas for further research.\*

\* Totten J and Goss T. The Impact of Payer Coverage and Reimbursement Policies on Off-Label Use of Anticancer Therapies. Oncology Issues. January/February 2006.

## *Oncology Practice Characteristics:*

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- Most survey respondents are physicians or practice managers/administrators.
  - Our sample is geographically diverse.
  - Most oncology practices surveyed are located in urban and suburban areas.
  - We surveyed a mix of oncology practice sizes.
  - More than half of oncology practices surveyed see more than 300 oncology patients per month.
- ➔ **The characteristics of the survey participants are representative of the general characteristics of ACCC's membership.**

Please refer to Appendix B for a detailed analysis of survey participant characteristics.

***Under Medicare Part B, coverage of off-label uses of anticancer medicines is required for—but not limited to—uses supported by certain drug compendia or medical literature.***

- Section 1861(t)(2)(B) of the Social Security Act (SSA) directs Medicare contractors to cover off-label uses of FDA-approved anticancer drugs and biologicals if supported by specified medical literature or drug compendia.
- In particular, a “medically accepted indication” includes any use that has been approved by the FDA, and includes another use of the drug if:

*“such use is supported by one or more citations which are included (or approved for inclusion) in one or more of the following compendia: the American Hospital Formulary Service-Drug Information, the American Medical Association Drug Evaluations, the United States Pharmacopoeia-Drug Information, and other authoritative compendia as identified by the Secretary, unless the Secretary has determined that the use is not medically appropriate or the use is identified as not indicated in one or more such compendia,”*

OR

*“the carrier involved determines, based upon guidance provided by the Secretary to carriers for determining accepted uses of drugs, that such use is medically accepted based on supportive clinical evidence in peer reviewed medical literature appearing in publications which have been identified for purposes of this subclause by the Secretary.”*

Source: Section 1861(t)(2)(B) of the Social Security Act

*The Medicare Benefit Policy Manual further details the Centers for Medicare and Medicaid Services' (CMS's) coverage policy for off-label use of anticancer therapies.*

- Per Section 50.4.2 of the Medicare Benefit Policy Manual, an off-label use of a drug is a use that is not included as an indication on the drug's label as approved by the FDA.
  - Off-label uses of FDA-approved anticancer drugs are covered by Medicare Part B if the contractor determines the use to be medically accepted, taking into consideration
    - major drug compendia,
    - authoritative medical literature, and/or
    - accepted standards of medical practice.
  - Section 50.4.5 of the Medicare Benefit Policy Manual instructs contractors not to deny coverage based solely on the lack of FDA approval if an indication for an anticancer drug meets one of the above criteria (and is not listed as “not indicated” in one of the major drug compendia).
- CMS recently has made policy changes to expand its list of Part B-recognized drug compendia, as well as its list of authoritative peer-reviewed literature.**

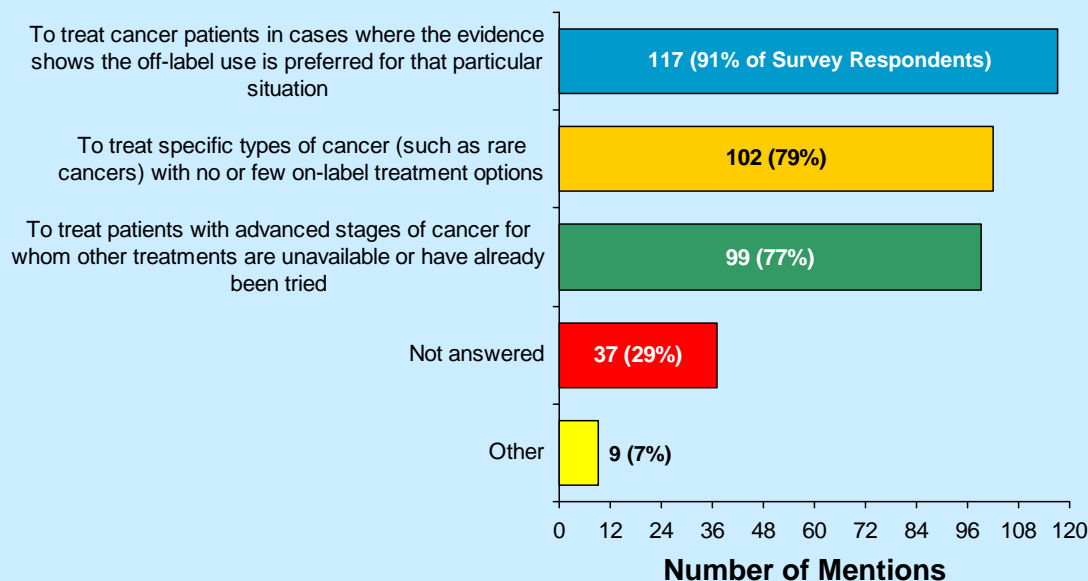
Source: Sections 50.4.2 and 50.4.5 of the Medicare Benefit Policy Manual

# *Key Findings*

## Role of Off-label Use

- Off-label use is at least somewhat important to 79 percent of oncology practices surveyed, and is extremely important to 50 percent of oncology practices.
- Oncology practices cite a variety of reasons for why off-label use of anticancer therapies is important to the treatment of their cancer patients.

### Most Frequently Cited Reasons for Why Off-Label Use of Anticancer Therapies Is Important to Oncology Practices' Treatment of Their Cancer Patients (N=129 Respondents)



## *Trends in Off-Label Use*

- Nearly half of the respondents report that their practice's frequency of off-label use of anticancer therapies has decreased over the past five years.
- Approximately 26 percent of respondents report that their practice's frequency of off-label use of anticancer therapies has increased over the past 5 years.
  - *This finding differs from Covance's 2005 survey, in which 42.5 percent of interviewees reported that their frequency of off-label use had increased over the previous 5 years. This potential shift in practice patterns should be further evaluated.*

## *Trends in Off-Label Use (cont'd)*

- Coverage and reimbursement challenges were the overwhelming reasons cited by oncology practices for their decreased use of anticancer therapies for off-label indications.

*"[Our frequency of off-label use of anticancer therapies has decreased over the past five years] mainly because of the lack of payment by Medicare and [private] insurance companies."*

**-- Oncology Practice Respondent**

- Oncology practices that report an increase in off-label use attribute their increased utilization to the availability of more drugs that have been shown to be effective in uses that are not yet FDA-approved.

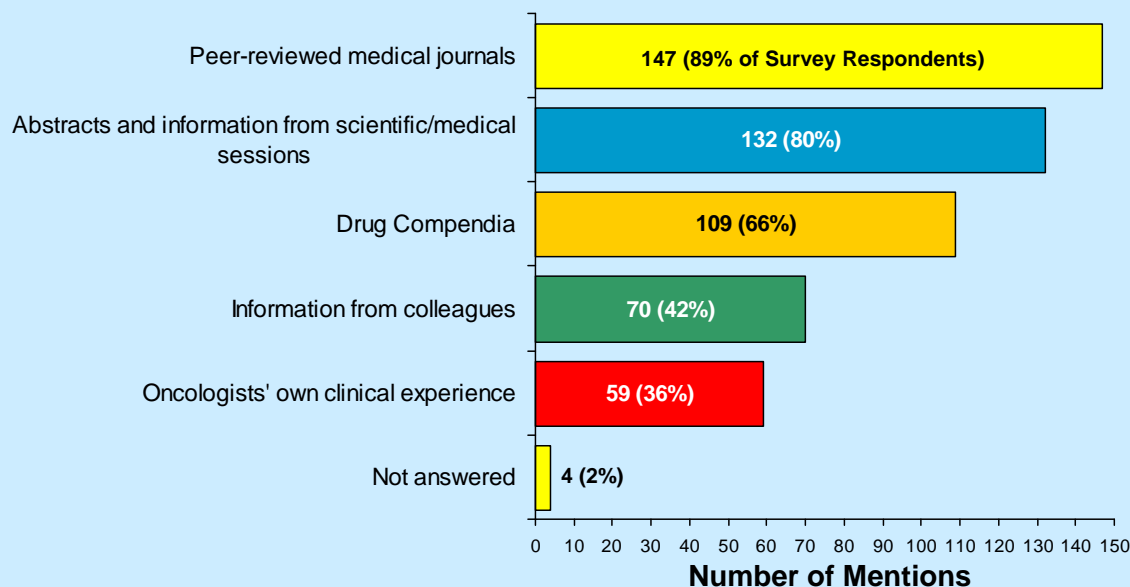
*"The speed of clinical research is faster than the approval process for including the drug in compendia listing. This plus the fact that patients are very enlightened and request access to drugs from their research."*

**-- Oncology Practice Respondent**

## *Information Sources Relied On for Off-Label Use*

- Oncology practices rely on a variety of sources of information to make off-label treatment decisions for anticancer therapies.
  - Peer-reviewed literature, abstracts and information from scientific sessions, and drug compendia are the three sources cited most frequently.

**Sources of Information Oncology Practices Rely on to Make Off-Label Treatment Decisions for Anticancer Therapies (N=165 Respondents)**



## *Importance of Information Sources for Off-Label Use*

- Eighty-one percent of oncology practices surveyed consider drug compendia at least somewhat important to their practice's use of off-label anticancer therapies.
- For 90 percent of respondents, peer-reviewed medical literature is somewhat important or extremely important.
- Ninety-one percent of respondents believe that there is a need for the Centers for Medicare and Medicaid Services (CMS) to recognize multiple compendia as a basis for covering off-label uses of anticancer therapies.\*

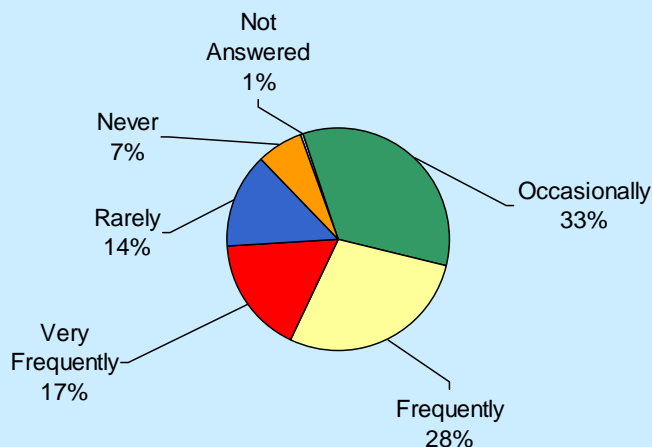
\* Our survey was deployed before CMS announced that it would recognize additional compendia (DRUGDEX, NCCN, and Clinical Pharmacology) for Part B off-label coverage. Although the policy context for this question has changed, the responses still indicate strong provider support for recognition of multiple compendia under Medicare Part B.

# Impact of Coverage and Payment Policy on Off-Label Prescribing

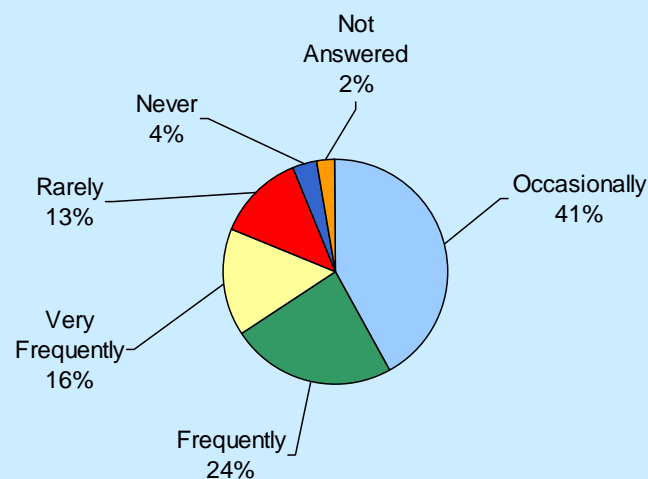
- Approximately 40 to 45 percent of respondents report that payer coverage and reimbursement policies (Medicare and private payers) cause them to frequently or very frequently alter their clinical decisionmaking.

How Often Payer Coverage and Reimbursement Policies for Off-Label Use of Anticancer Drugs Cause Oncology Practices to Alter Their Clinical Decisionmaking (N=165 Respondents)

## Local Medicare Contractors



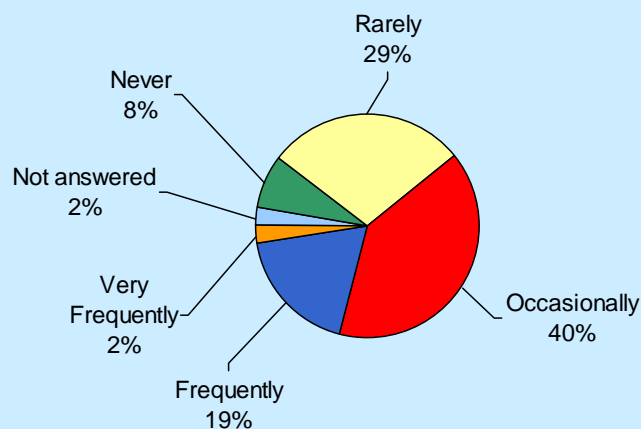
## Private Payers



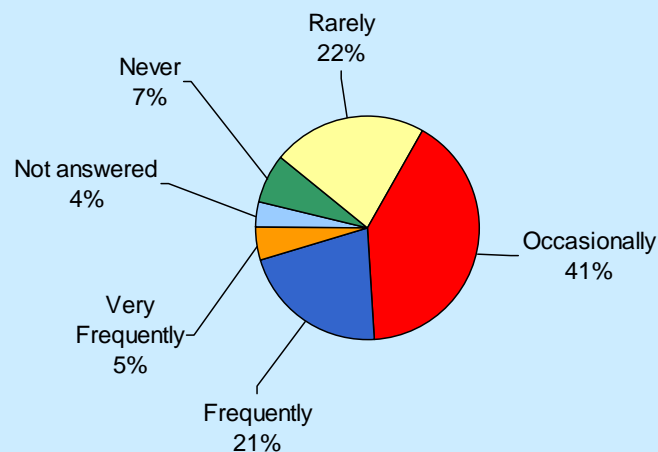
## Impact of Coverage and Payment Policy on Off-Label Prescribing (cont'd)

- When asked how often coverage denials involve off-label uses of medicines that are supported by drug compendia or peer-reviewed medical literature, at least 20 percent of survey respondents reported that this occurs frequently or very frequently.

**How Often Coverage Denials of Off-Label Uses of Anticancer Drugs Involve an Off-Label Use Listed in Drug Compendia as an “Accepted” Indication**  
N=165 Respondents



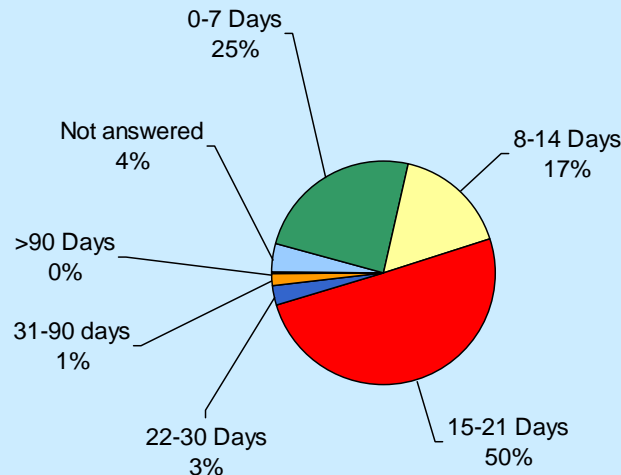
**How Often Coverage Denials of Off-Label Uses of Anticancer Drugs Involve an Off-Label Use Supported by Peer-Reviewed Medical Literature (but not compendia-listed)**  
N=165 Respondents



## *Impact of Coverage and Payment Policy on Off-Label Prescribing (cont'd)*

- Oncology practices respond in a variety of ways when faced with coverage restrictions on off-label uses.
  - Common responses include altering the drug regimen, seeking assistance from patient assistance programs, changing the treatment setting, and appealing a denial.
- For 54 percent of practices, off-label coverage and reimbursement policies result in treatment delays of 15 days or longer.

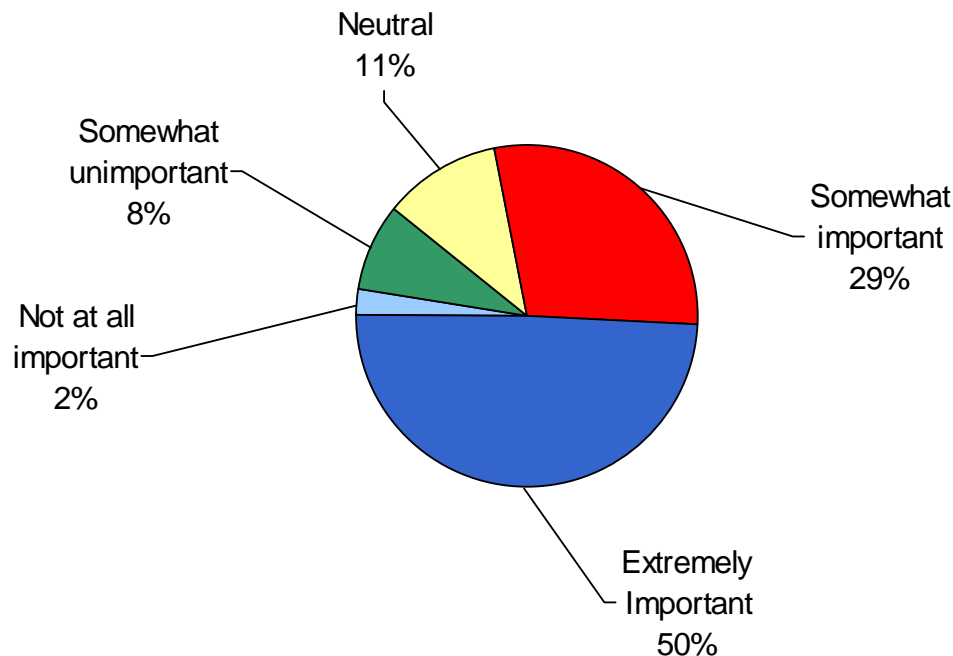
Typical Treatment Delay in Number of Days (N=151 Respondents)



# *Current Off-Label Use of Anticancer Therapies*

*Off-label use is at least somewhat important to 79 percent of oncology practices surveyed, and is extremely important to 50 percent of oncology practices.*

**Importance of Off-Label Use of Anticancer Therapies to the Treatment of Oncology Practices' Cancer Patients\*  
N=165 Respondents**

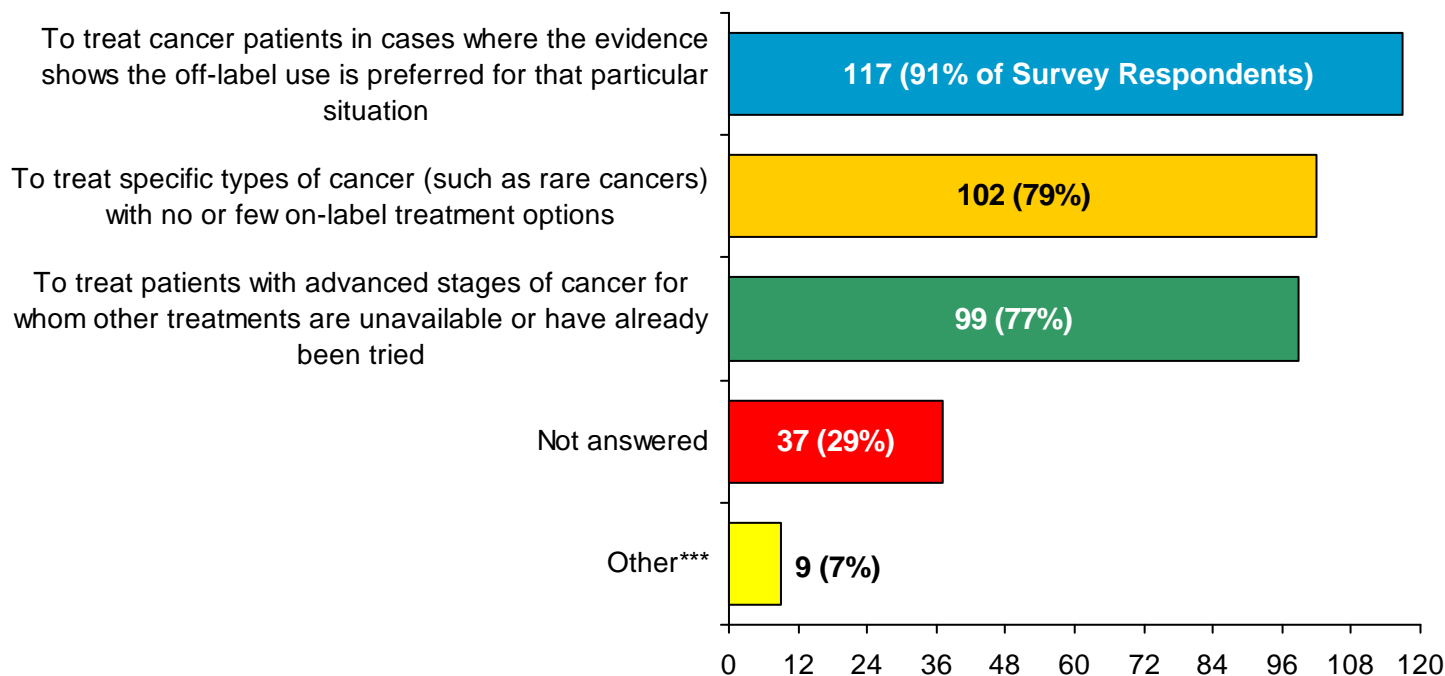


\* Categories represent multiple choice responses.

# *Oncology practices cite a variety of reasons for why off-label use of anticancer therapies is important to the treatment of their cancer patients.*

## **Most Frequently Cited Reasons for Why Off-Label Use of Anticancer Therapies Is Important to Oncology Practices' Treatment of Their Cancer Patients\***

**N=129 Respondents\*\***



**Number of Mentions**

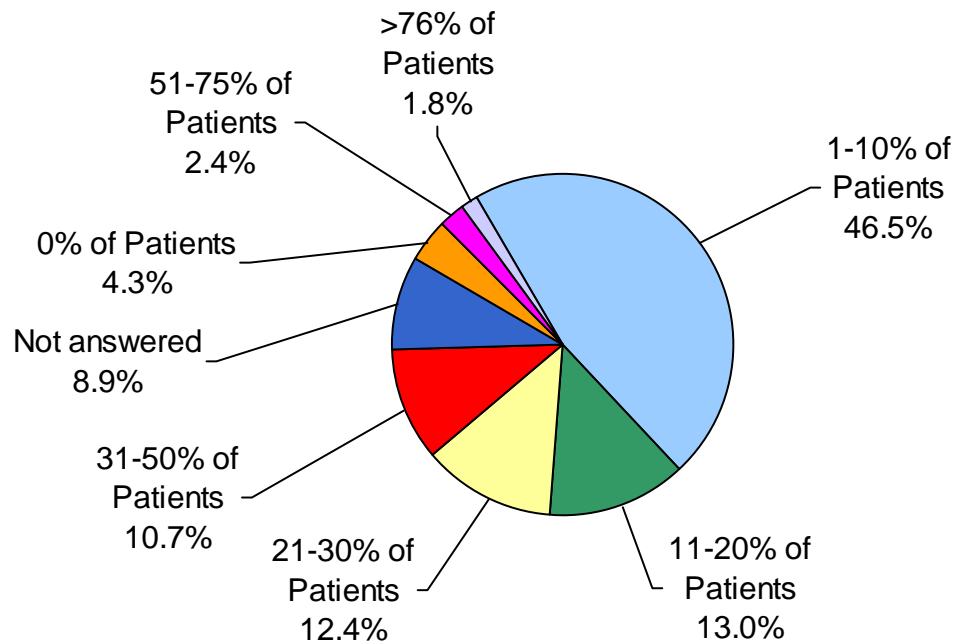
\* Categories represent multiple choice responses; respondents could select all that apply.

\*\* This question was answered only by those oncology practices that reported that off-label use of anticancer therapies is "Somewhat Important" or "Extremely Important" to the treatment of their cancer patients.

\*\*\* Other includes: "Published studies suggest efficacy of an off-label regimen"; "Lag time of FDA approvals"; "Some off-label therapies are less expensive than FDA-approved therapies"; and "Side effects of labeled treatments are sometimes contraindicated."

# *Most oncology practices prescribe anticancer drugs for off-label uses for at least some of their patients.*

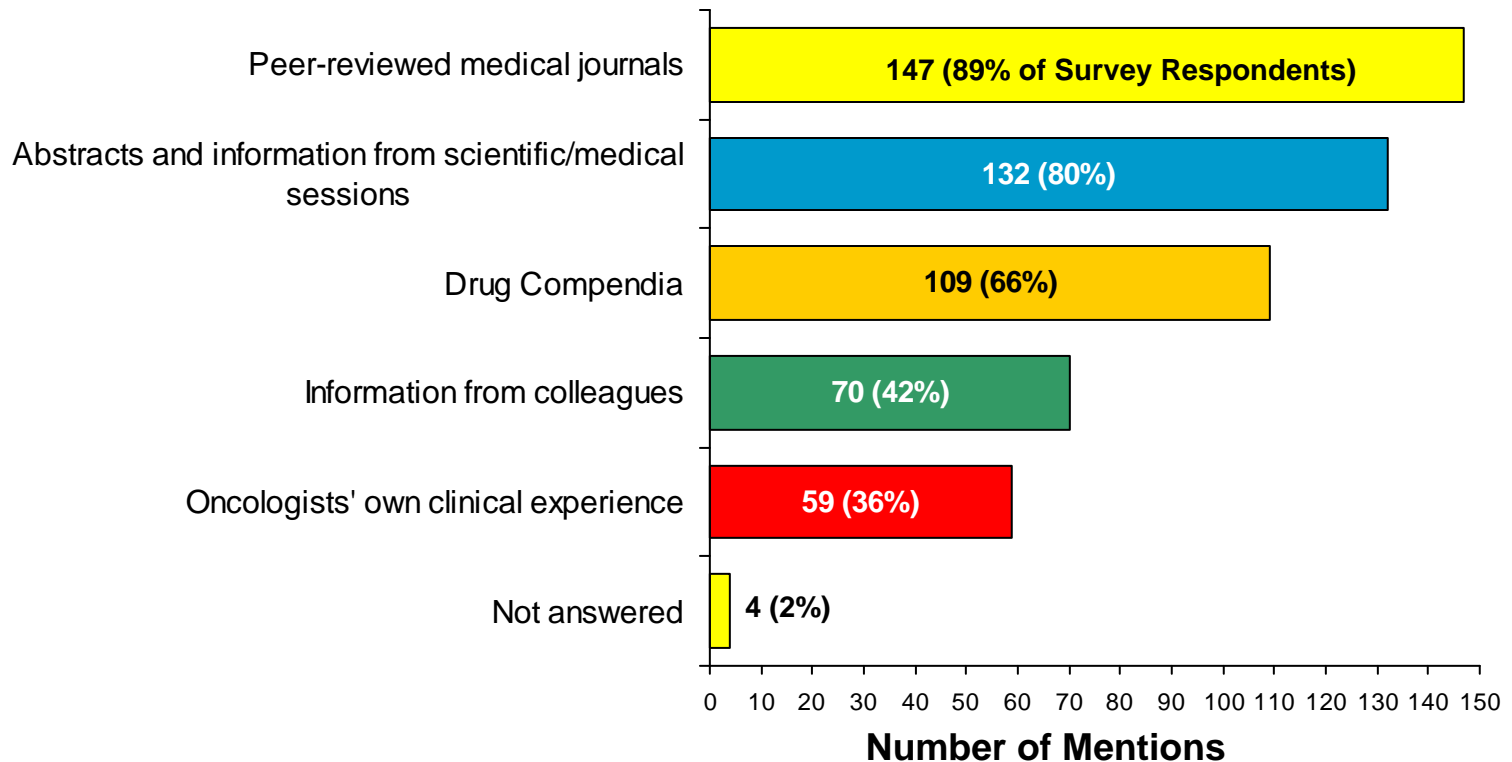
**Percentage of Oncology Practices' Cancer Patients that Are Prescribed at Least One Anticancer Drug for an Off-Label Use During the Course of Care\*  
N=165 Respondents**



\* Increments created based on free-form numerical responses.

# *Oncology practices rely on a variety of sources of information to make off-label treatment decisions for anticancer therapies.*

**Sources of Information Oncology Practices Rely on to Make Off-Label Treatment Decisions for Anticancer Therapies\***  
**N=165 Respondents**

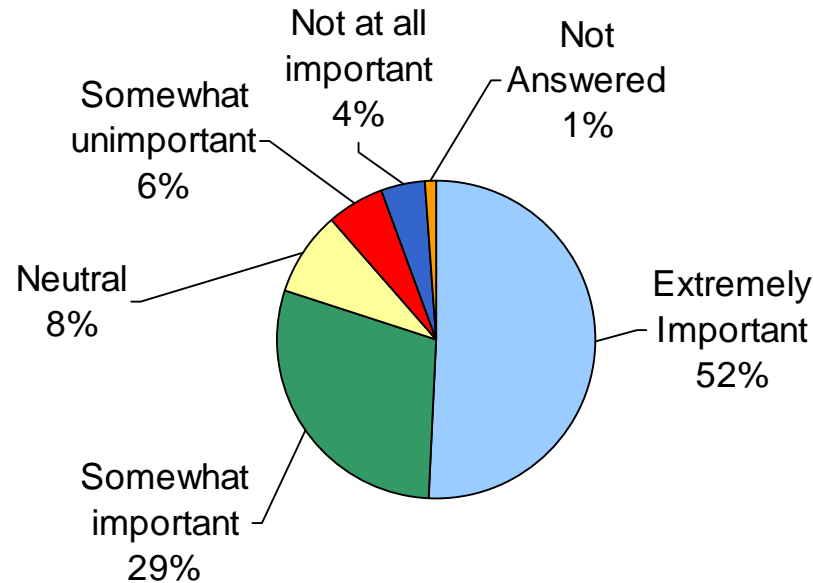


\* Categories represent multiple choice responses; respondents could select all that apply.

*More than half of oncology practices surveyed consider drug compendia extremely important to their practice's use of off-label anticancer therapies; 81 percent consider drug compendia at least somewhat important.*

**Importance of Drug Compendia to Oncology Practices' Use of  
Label Anticancer Therapies\*  
N=165 Respondents**

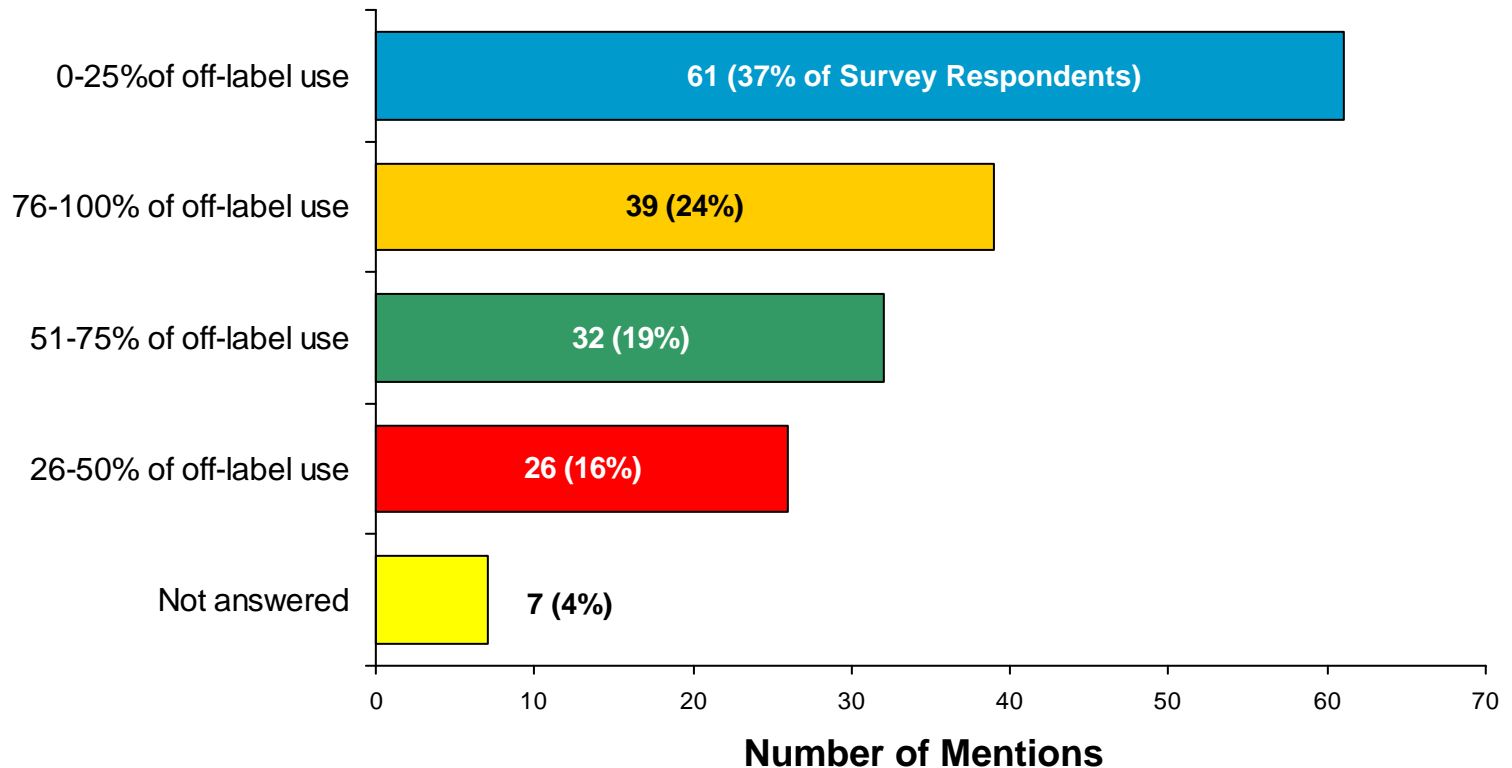
Off-



\* Categories represent multiple choice responses.

# *A significant portion of off-label use is supported by information sources other than drug compendia.*

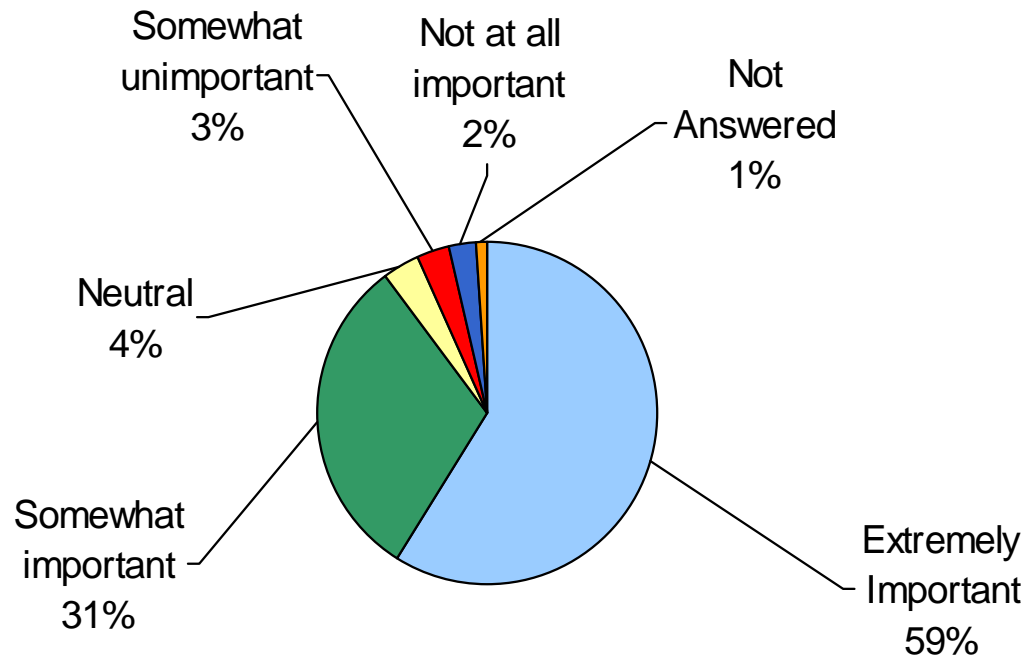
**Percentage of Oncology Practices' Off-Label Use that Is Supported by Drug Compendia\***  
**N=165 Respondents**



\* Categories represent multiple choice responses.

*For 90 percent of respondents, peer-reviewed medical literature is somewhat important or extremely important.*

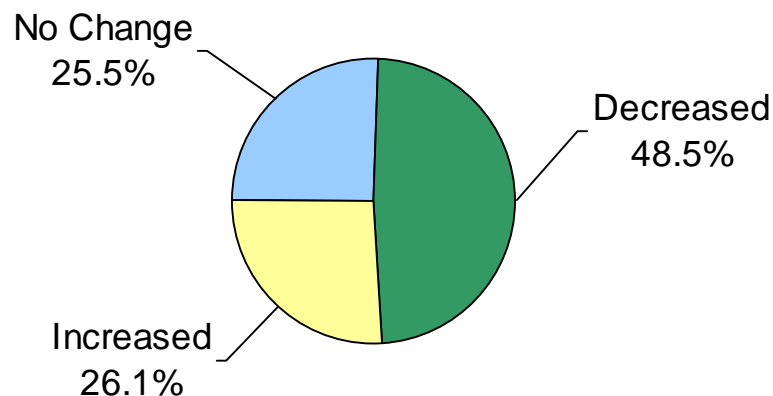
**Importance of Peer-Reviewed Medical Literature to Oncology Practices' Use of Off-Label Anticancer Therapies\*  
N=165 Respondents**



\* Categories represent multiple choice responses.

*Nearly half of the respondents report that their practice's frequency of off-label use of anticancer therapies has decreased over the past five years.*

**How Oncology Practices' Frequency of Off-Label Use Has Changed  
Over the Past Five Years\*  
N=165 Respondents**

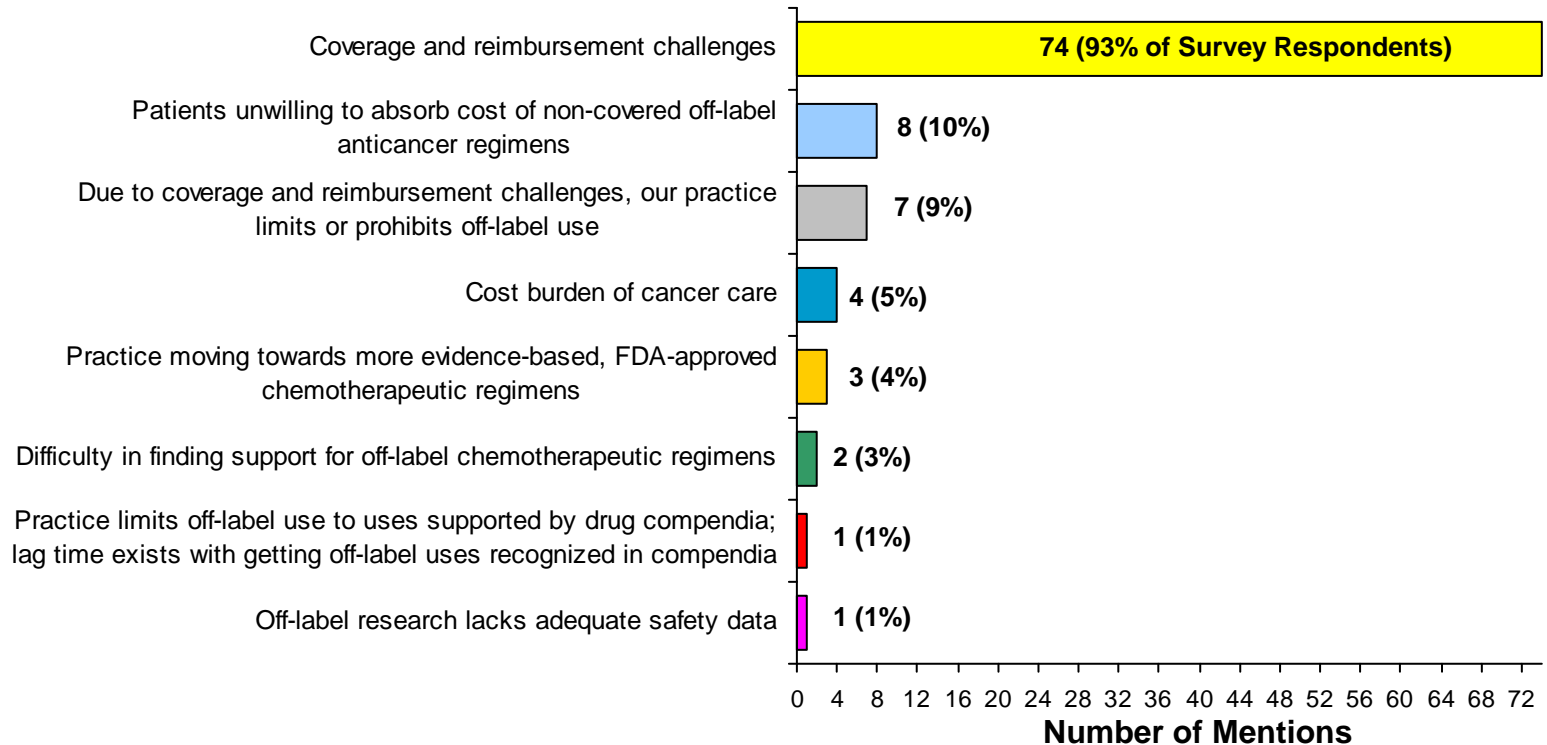


→ This finding differs from Covance's 2005 survey, in which 42.5 percent of interviewees reported that their frequency of off-label use had increased over the previous 5 years. This potential shift in practice patterns should be further evaluated.

\* Categories represent multiple choice responses.

*Coverage and reimbursement challenges were the overwhelming reasons cited by oncology practices for their decreased use of anticancer therapies for off-label indications.*

**Reasons Cited for Why the Frequency of Off-Label Use of Anticancer Therapies Has Decreased Over the Past Five Years\*  
N=80 Respondents\*\***



\* Data are derived from free-form responses. Multiple responses per respondent may be represented.

\*\* This question was answered only by those oncology practices that reported that the frequency of their off-label use has decreased over the past five years.

## *Coverage and reimbursement challenges were the overwhelming reasons cited by oncology practices for their decreased use of anticancer therapies for off-label indications (cont'd).*

*“Our frequency of off-label use of anticancer therapies has decreased over the past five years] mainly because of the lack of payment by Medicare and [private] insurance companies.”*

**-- Oncology Practice Respondent**

*“Our practice has lost a significant amount of money in previous years by using off label anticancer therapies that were not reimbursed, even after having obtained pre-authorization by payers. After this financial situation was reviewed, our Board of Managers established a policy that prohibits off label use of any drug.”*

**-- Oncology Practice Respondent**

*“[We have decreased our frequency of off-label prescribing due to] reimbursement issues. We have negated nearly all off label use because of the hassles of billing and recouping the drug charges and associated infusion codes. We used to be able to send in appeals with supporting data and eventually get paid. Now with time/value of money alone and cost of tracking and documentation it is highly discouraged.”*

**-- Oncology Practice Respondent**

*“[Off-label uses of anticancer drugs] do not get [reimbursed] DESPITE providing all necessary journals and abstracts; the insurers continue to call it experimental and deny claims. Thus our practice is out money for the medication, and the patient is balanced billed and cannot afford it. It is SO unfair.”*

**-- Oncology Practice Respondent**

## *Coverage and reimbursement challenges were the overwhelming reasons cited by oncology practices for their decreased use of anticancer therapies for off-label indications (cont'd).*

*"It has become much more difficult to get coverage by [private insurers] and Medicare for off label use of chemotherapy. I have even had cases where the standard treatment for a cancer, e.g., metastatic gastroesophageal adenocarcinoma, has been turned down by insurers because the chemotherapy drugs do not have that specific disease listed in their FDA approvals. Treatment was denied even though it was indicated by [the American Society of Clinical Oncology (ASCO)] and NCCN, and the insurance reviewer did not care that we had multiple publications showing benefit to this therapy, and we had a second opinion from a tertiary university cancer center that also endorsed the therapy. At what point do you tell the patient to just give up? In this case of GE junction cancer, the patient left his job, went on Medicaid, got the therapy, and responded. He is alive and well now 1 year later. We have only had partial payment."*

**-- Oncology Practice Respondent**

*"We only give therapies that are covered which requires the compendia if not FDA approval. Most health plans are denying non compendia listed therapies. Rarely are they approved... We are in a highly managed care environment for PPO\* and HMO\* and Medicare membership."*

**-- Oncology Practice Respondent**

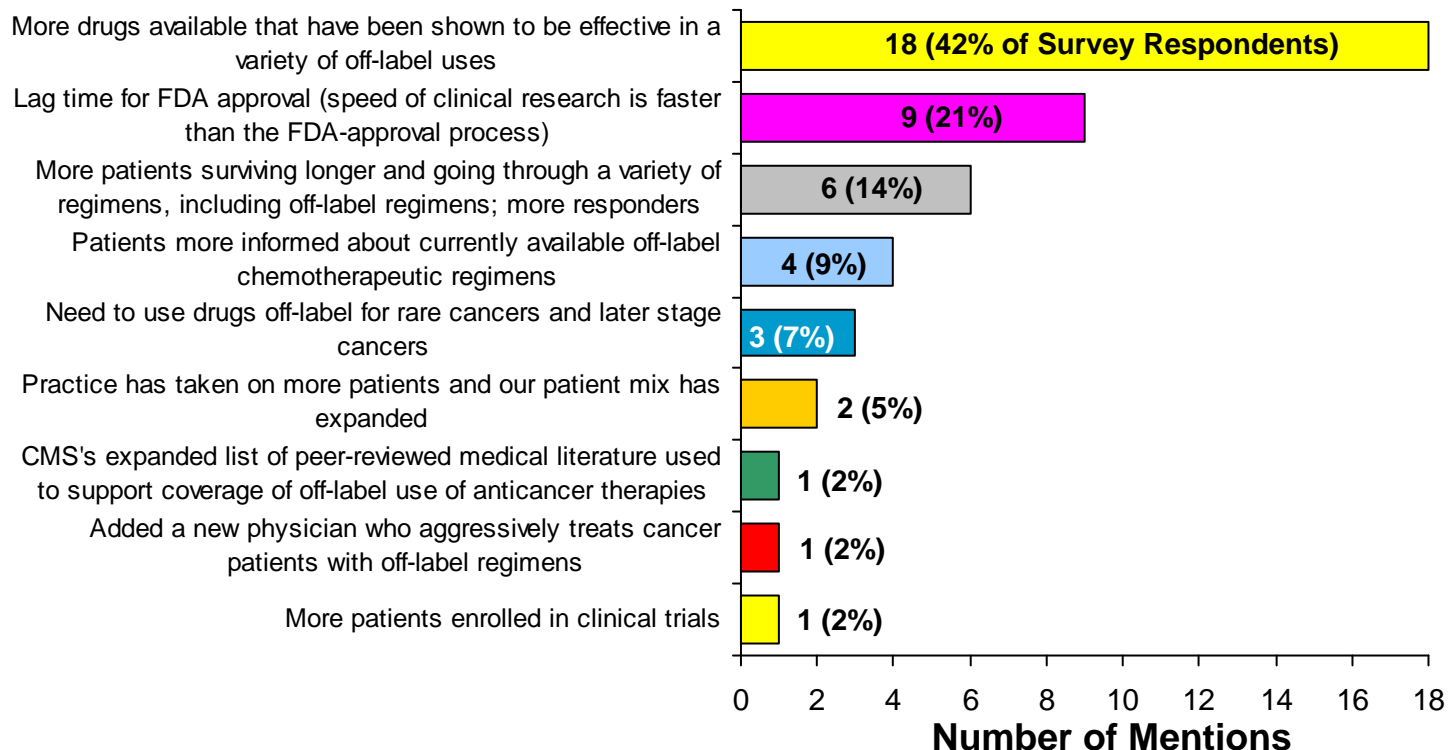
*"Our practice has basically stopped the use of off-label therapies due to the difficulties in appeals, reimbursement issues, and with all the cuts in the past five years we must utilize the staff we have more effectively."*

**-- Oncology Practice Respondent**

\* Preferred provider organization (PPO); Health maintenance organization (HMO)

***Oncology practices that report an increase in off-label use attribute their increased utilization to the availability of more drugs that have been shown to be effective in uses that are not yet FDA-approved.***

**Reasons Cited for Why the Frequency of Off-Label Use of Anticancer Therapies Has Increased Over the Past Five Years\*  
N=43 Respondents\*\***



\* Data are derived from free-form responses. Multiple responses per respondent may be represented.

\*\* This question was answered only by those oncology practices that reported that the frequency of their off-label use has increased over the past five years.

***Oncology practices that report an increase in off-label use attribute their increased utilization to the availability of more drugs that have been shown to be effective in uses that are not yet FDA-approved (cont'd).***

*“More drugs are available that have been shown to be effective in tumors but not submitted to FDA for that specific situation. Even if this is done by the drug company it may lag years behind scientific studies of efficacy.”*

**-- Oncology Practice Respondent**

*“The speed of clinical research is faster than the approval process for including the drug in compendia listing. This plus the fact that patients are very enlightened and request access to drugs from their research.”*

**-- Oncology Practice Respondent**

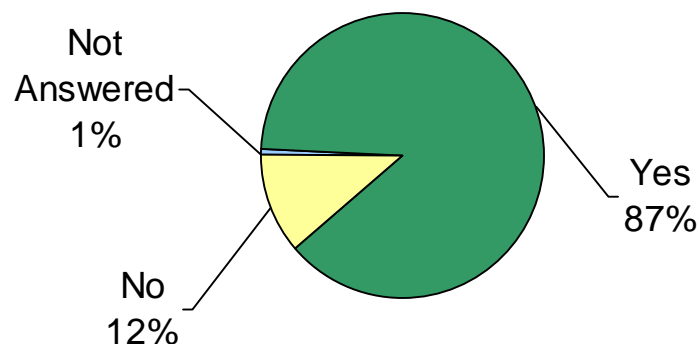
*“Over the past several years, our practice has taken on more patients, our patient mix has expanded, and pharmaceutical availability has increased. We look at every available resource to provide the best possible care to our patients. Off-label use is a very small way to contribute to this cause. The use of this method is still fairly new, but our physicians are open to new therapeutic solutions.”*

**-- Oncology Practice Respondent**

# *Coverage and Reimbursement of Off-Label Drug Use*

***Most oncology practice respondents are familiar with their local Medicare contractors' and private insurers' policies governing off-label use of anticancer therapies.\****

**Are you familiar with your local Medicare contractors' and private insurers' policies governing off-label use of anticancer therapies? \*\***  
**N=165 Respondents**



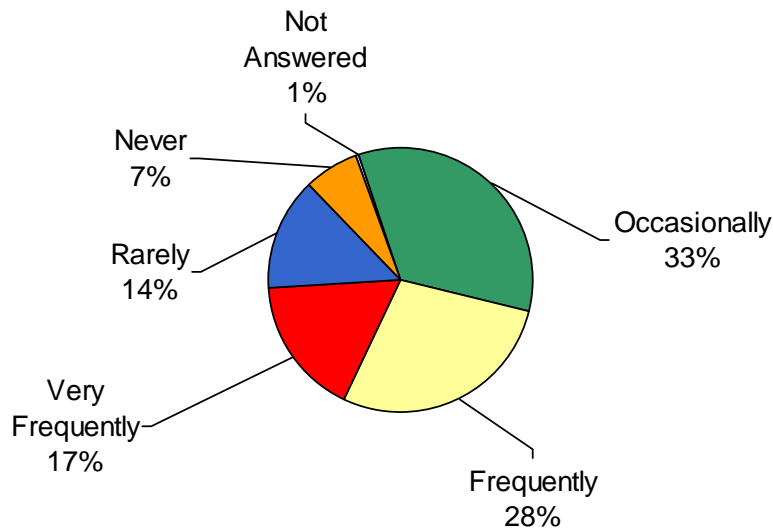
\* Survey responses may be biased, as individuals who are familiar with Medicare and private payer coverage and reimbursement policies for off-label uses of anticancer therapies may have been more likely to participate in our survey.

\*\* Categories represent multiple choice responses.

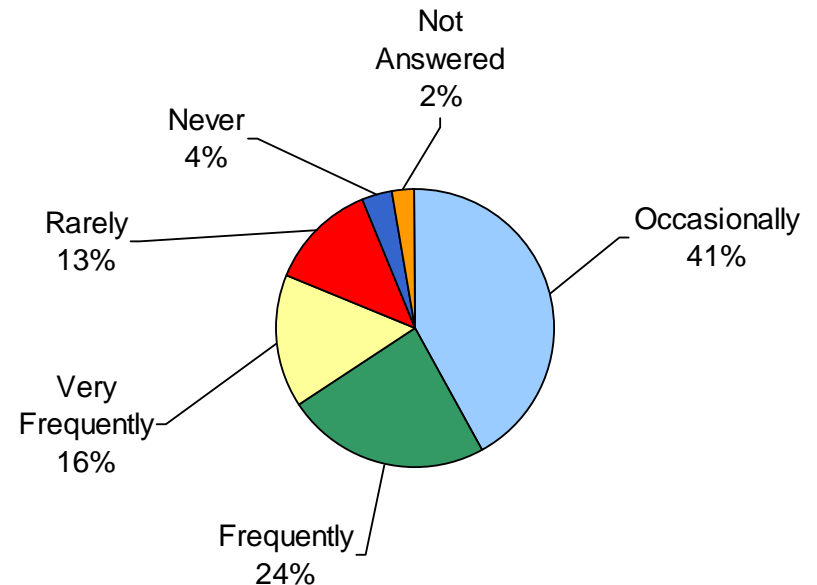
*Approximately 40 to 45 percent of respondents report that payer coverage and reimbursement policies (Medicare and private payers) for off-label use of anticancer drugs cause them to at least occasionally alter their clinical decisionmaking.*

**How Often Payer Coverage and Reimbursement Policies for Off-Label Use of Anticancer Drugs Cause Oncology Practices to Alter Their Clinical Decisionmaking\*  
N=165 Respondents**

**Local Medicare Contractors**



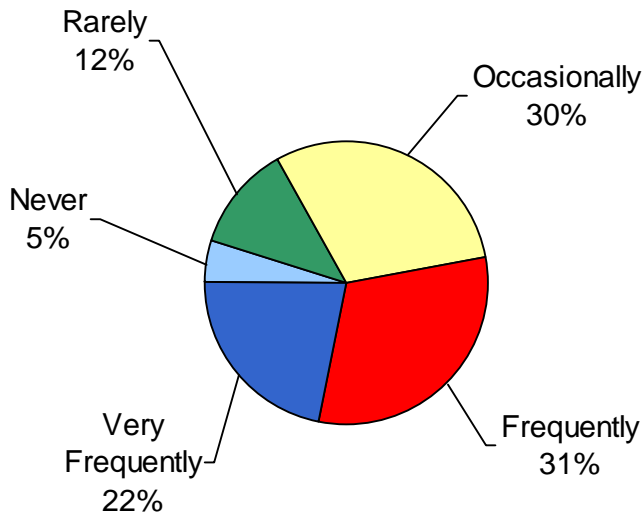
**Private Payers**



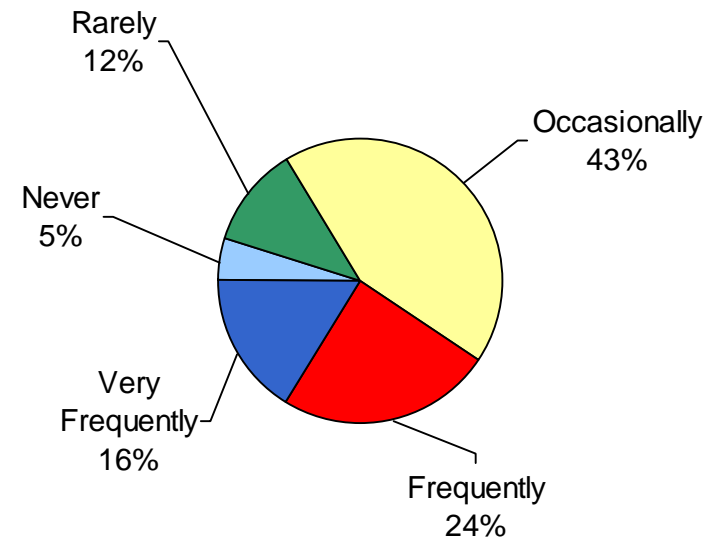
*More than half of respondents report that local Medicare contractors' coverage and reimbursement policies frequently or very frequently restrict their practices' off-label use of anticancer therapies; 40 percent report that private payers frequently or very frequently restrict off-label use.*

**How Often Payer Coverage and Reimbursement Policies Restrict Oncology Practices' Off-Label Use of Anticancer Drugs\***  
**N=165 Respondents**

**Local Medicare Contractors**



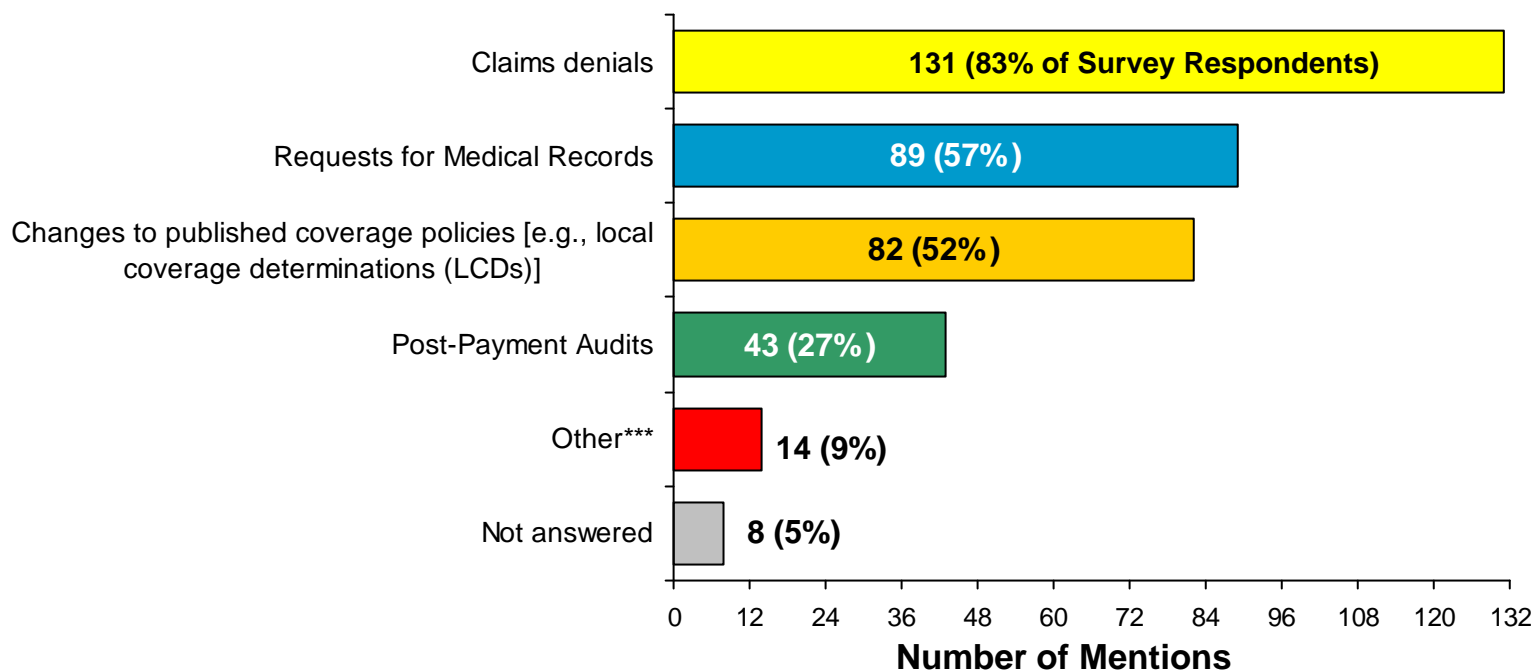
**Private Payers**



\* Categories represent multiple choice responses.

*Claims denials are the primary method Medicare contractors have used to become more restrictive with coverage and reimbursement for off-label uses of anticancer drugs.*

**Methods Medicare Contractors Have Used to Become More Restrictive with Coverage and Reimbursement for Off-Label Uses of Anticancer Drugs\*  
N=157 Respondents\*\***



\* Categories represent multiple choice responses; respondents could select all that apply.

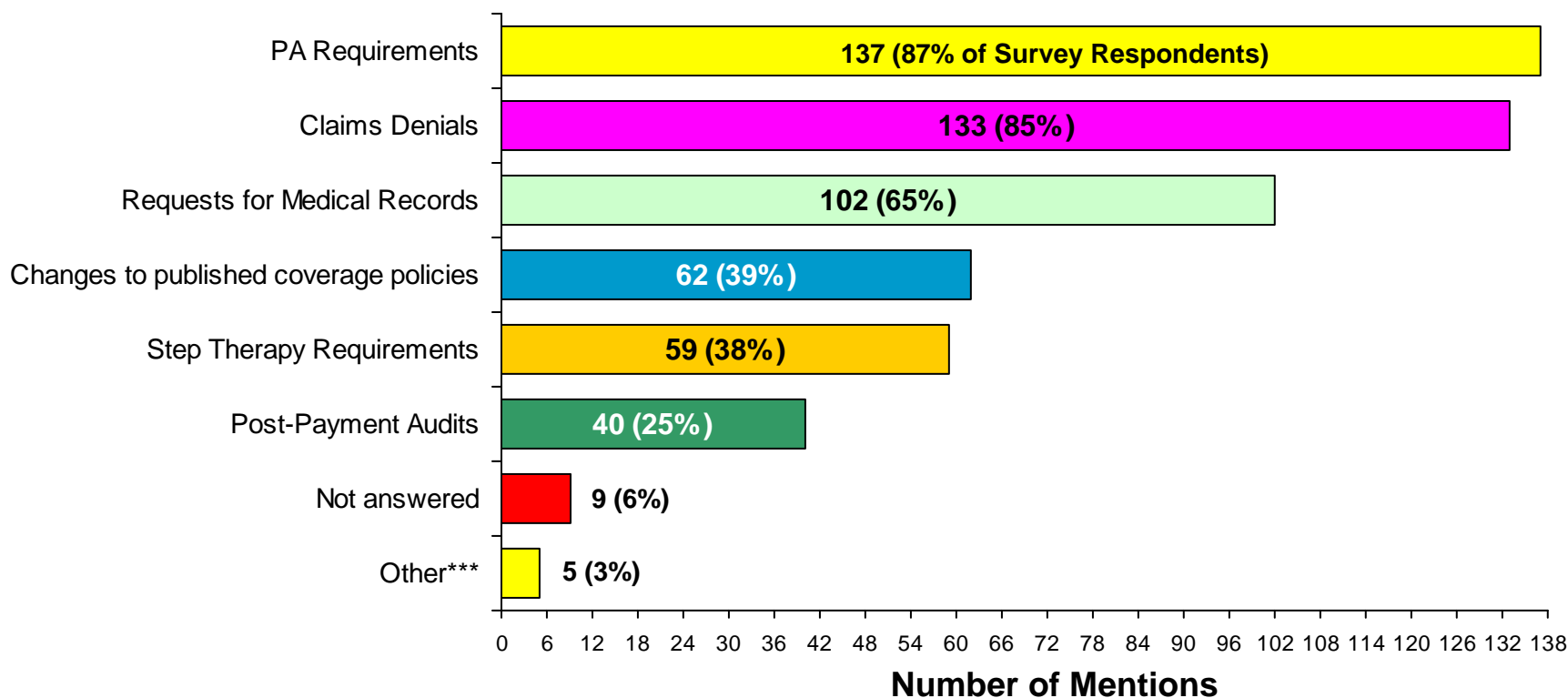
\*\* This question was answered only by those oncology practices that reported that Medicare contractors' coverage and reimbursement policies "rarely", "occasionally", "frequently", or "very frequently" restrict their practices' off-label use of anticancer drugs.

\*\*\* Other includes: "Delays coverage and reimbursement for off-label use of anticancer therapies"; "Does not know"; "Do not effectively communicate coverage and reimbursement policies"; "Requests peer-reviewed medical literature"; and "Requires letter of medical necessity."

***PA, claims denials, and requests for medical records are the primary methods private payers have used to become more restrictive with coverage and reimbursement for off-label uses of anticancer drugs.***

**Methods Private Payers Have Used to Become More Restrictive with Coverage and Reimbursement for Off-Label Uses of Anticancer Drugs\***

**N=157 Respondents\*\***



\* Categories represent multiple choice responses; respondents could select all that apply.

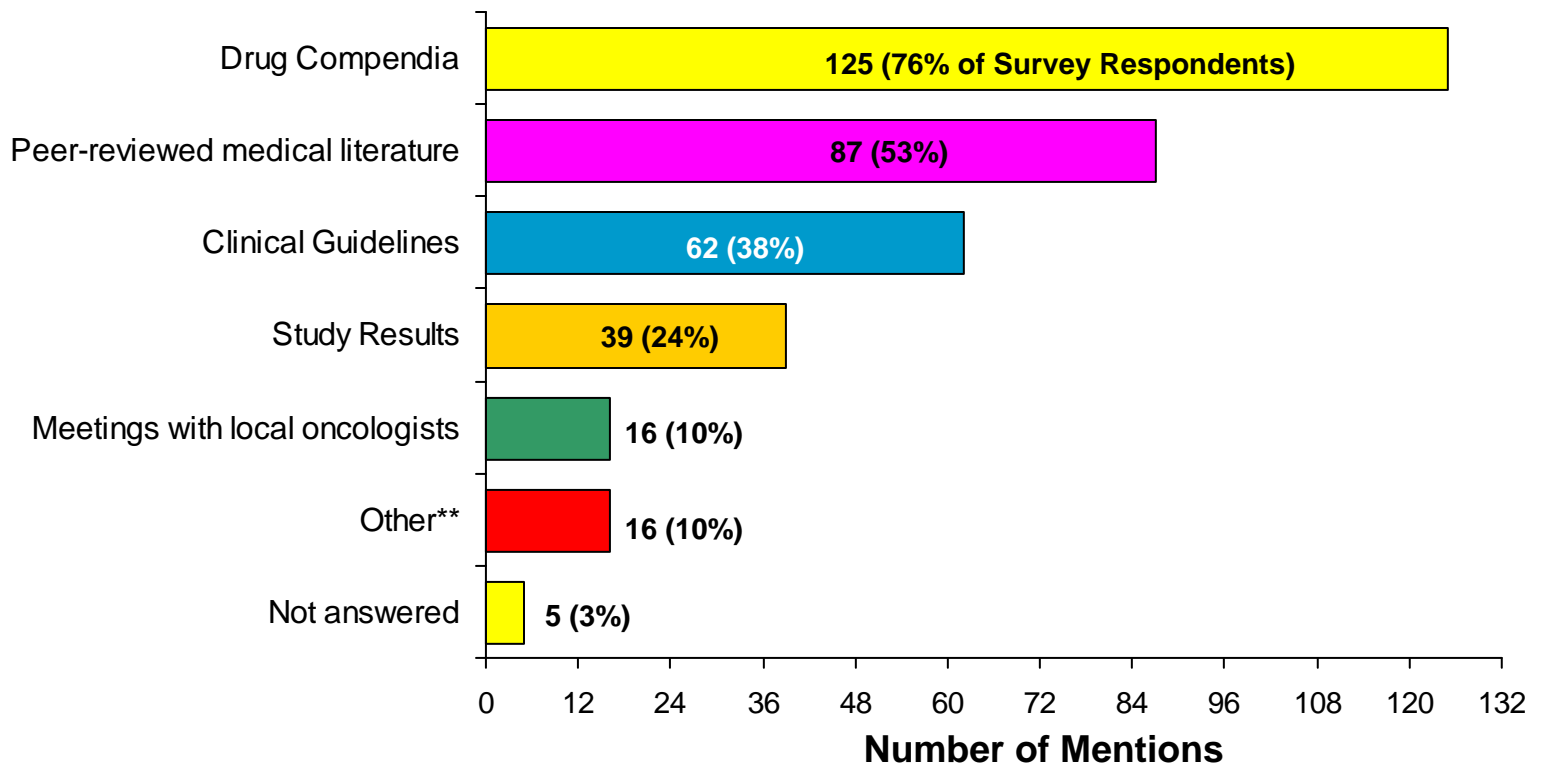
\*\* This question was answered only by those oncology practices that reported that private payers' coverage and reimbursement policies "rarely", "occasionally", "frequently", or "very frequently" restrict their practices' off-label use of anticancer drugs.

\*\*\* Other includes: "Delays coverage and reimbursement for off-label uses of anticancer therapies" and "Various other methods."

*Drug compendia are the primary sources of information that Medicare contractors use to support coverage and reimbursement for off-label uses of anticancer drugs.*

**Sources Medicare Contractors Use to Support Coverage and Reimbursement for Off-Label Uses of Anticancer Drugs\***

**N=165 Respondents**



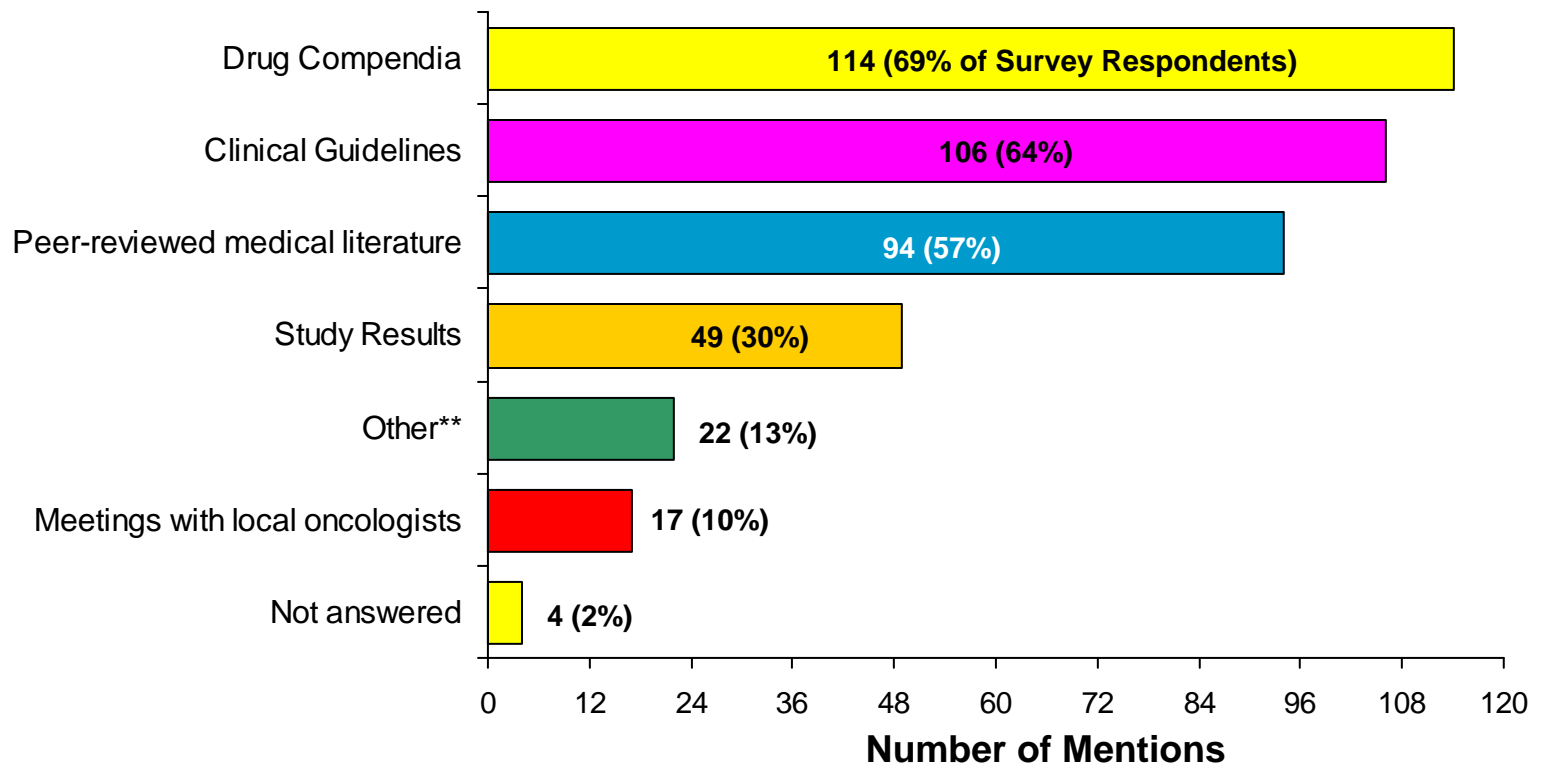
\* Categories represent multiple choice responses; respondents could select all that apply.

\*\* Other includes: "Does not know"; "Local Medicare contractor coverage policy"; and "Physician reviewer."

*Although they rely on drug compendia, private payers place almost equal emphasis on clinical guidelines and peer-reviewed medical literature to support coverage and reimbursement for off-label uses of anticancer drugs.*

### Sources Private Payers Use to Support Coverage and Reimbursement for Off-Label Uses of Anticancer Drugs\*

N=165 Respondents

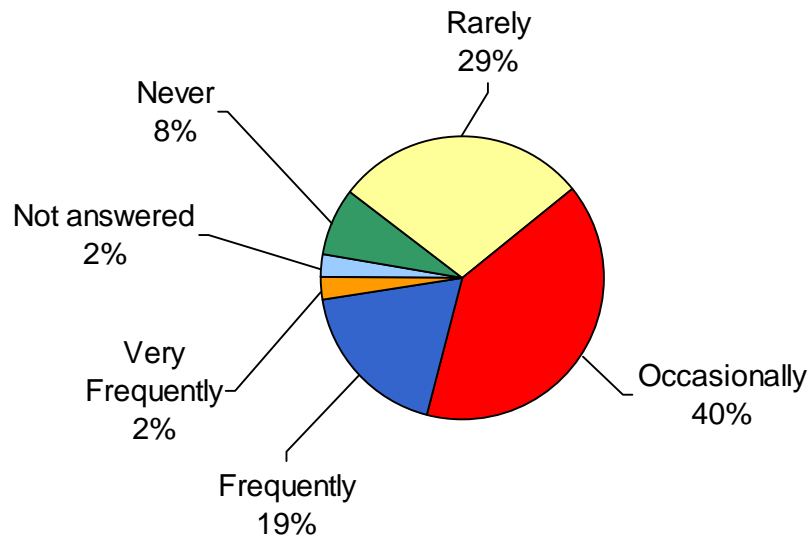


\* Categories represent multiple choice responses; respondents could select all that apply.

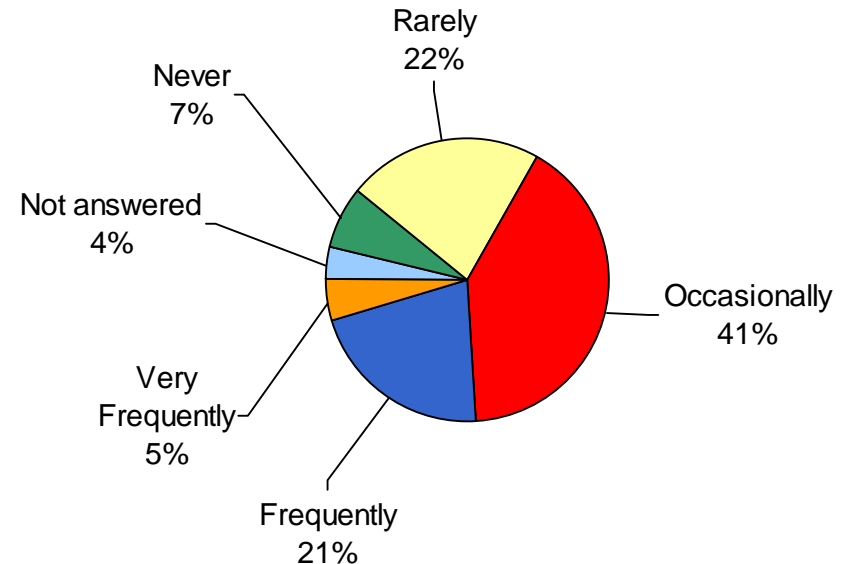
\*\* Other includes: "Does not know"; "Medical director discretion"; "Physician consultation with the plan"; and "Medicare criteria."

***At least 20 percent of oncology practices report that denials of coverage for off-label uses of anticancer drugs frequently or very frequently involve drug indications supported by compendia or peer-reviewed literature.***

**How Often Coverage Denials of Off-Label Uses of Anticancer Drugs Involve an Off-Label Use Listed in Drug Compendia as an “Accepted” Indication\*  
N=165 Respondents**



**How Often Coverage Denials of Off-Label Uses of Anticancer Drugs Involve an Off-Label Use Supported by Peer-Reviewed Medical Literature (but not compendia-listed)\*  
N=165 Respondents**

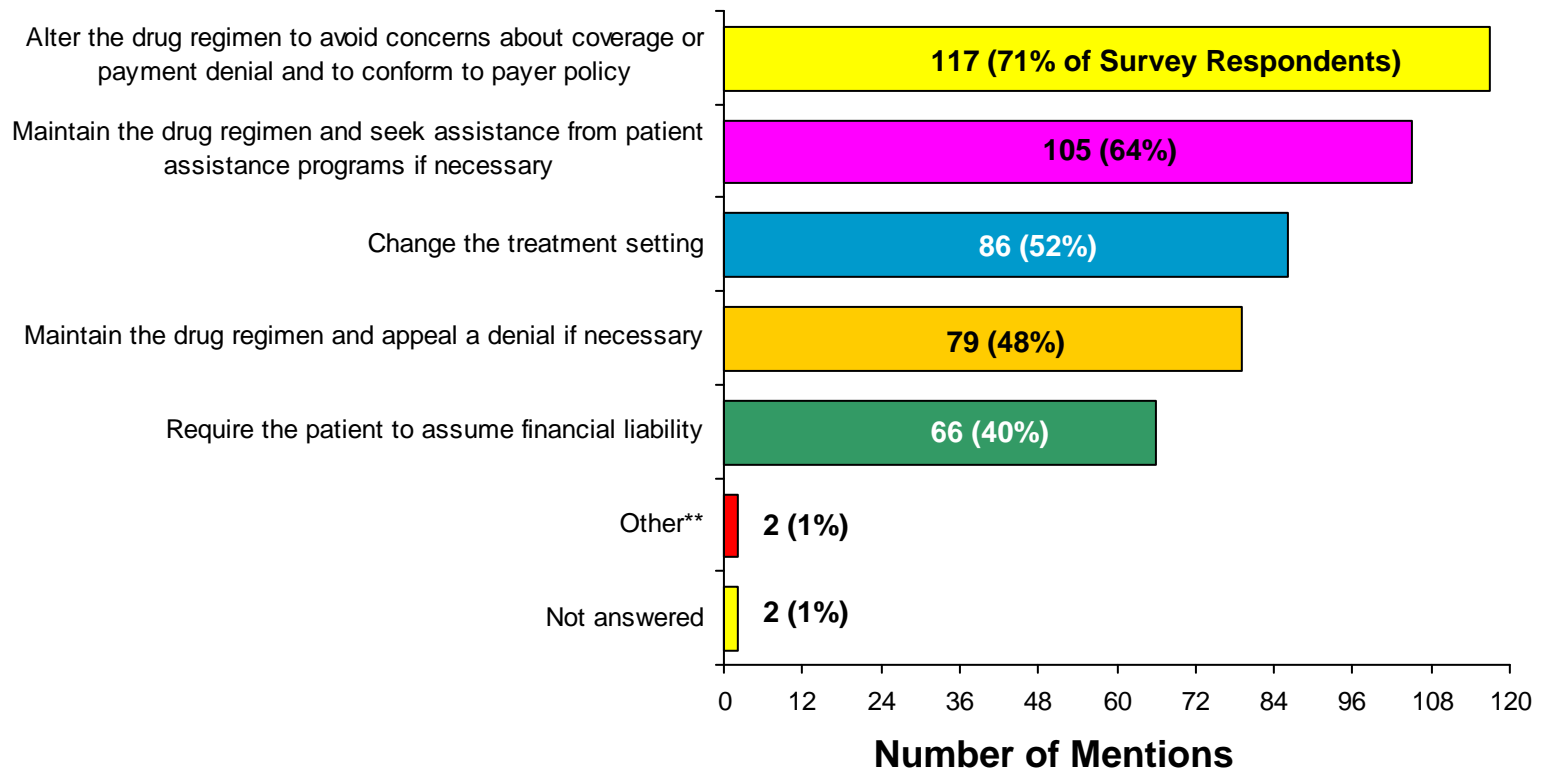


\* Categories represent multiple choice responses.

# *Oncology practices respond in a variety of ways when faced with restrictive off-label payer coverage policies.*

## How Oncology Practices Usually Respond When Payer Coverage and Reimbursement Policies for Particular Off-Label Uses of an Anticancer Drug Are Restrictive\*

N=165 Respondents



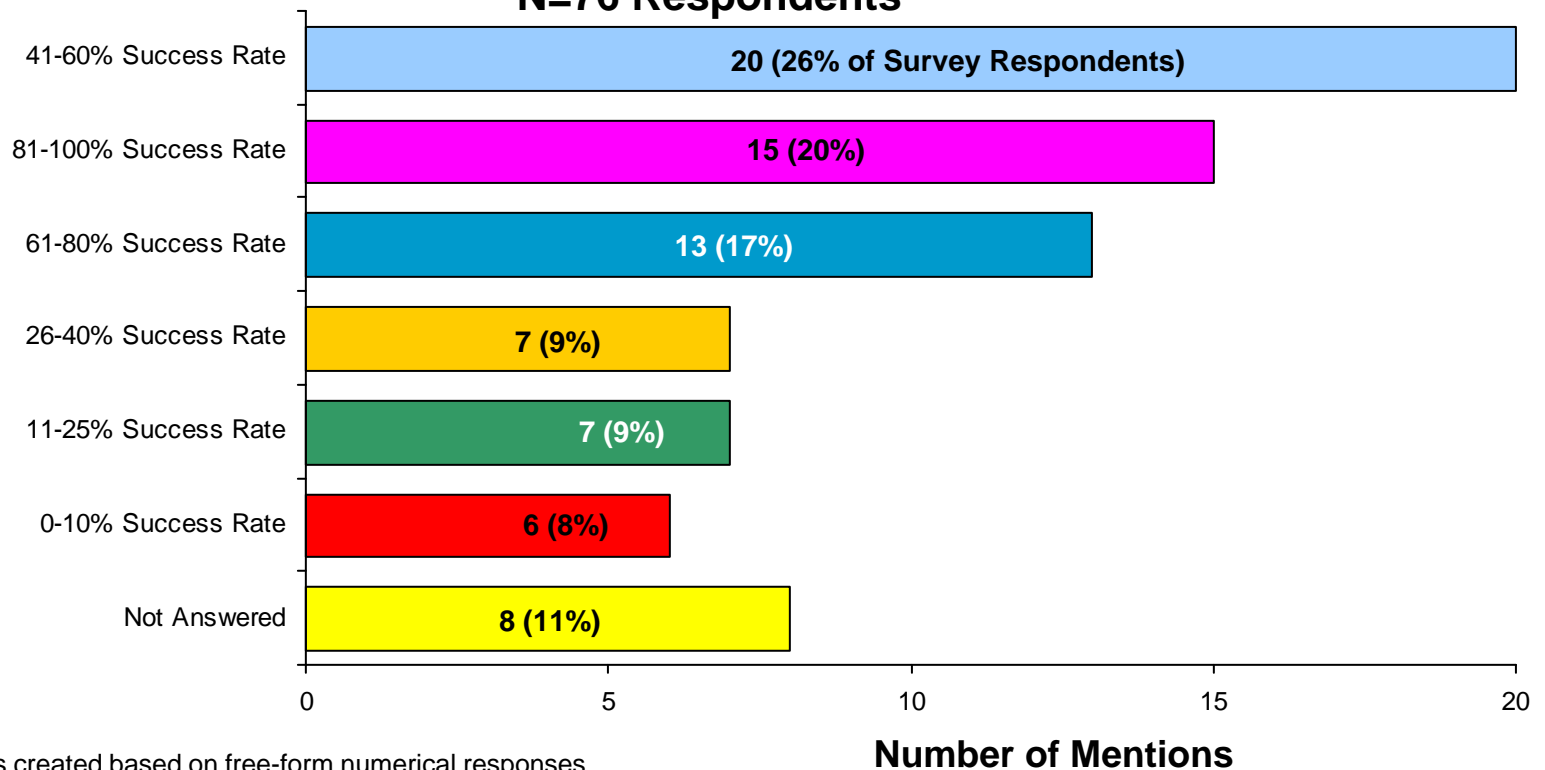
\* Categories represent multiple choice responses; respondents could select all that apply.

\*\* Other includes: "Ask manufacturer for replacement drug."

# *More than half of oncology practices have a success rate of 60 percent or less when appealing denied claims for off-label uses of anticancer drugs.*

## **Oncology Practices' Rate of Success (as a Percentage) with Appeals of Denied Claims for Off-Label Uses of Anticancer Drugs\***

**N=76 Respondents\*\***

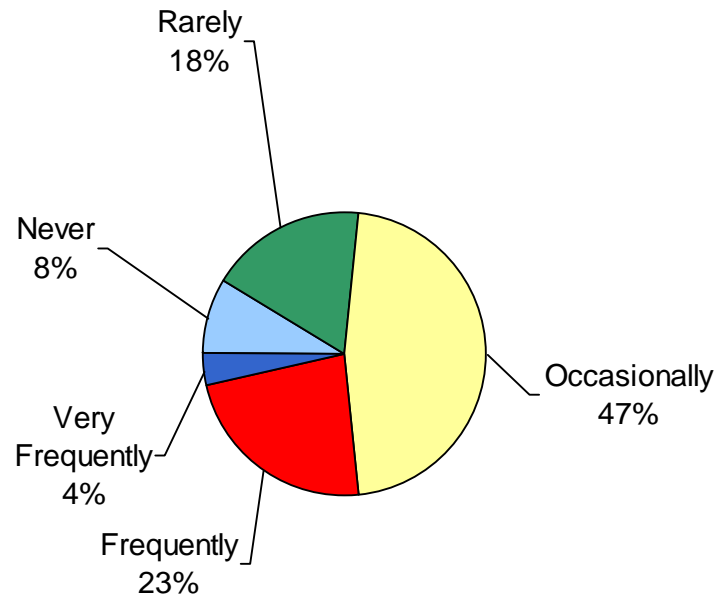


\* Increments created based on free-form numerical responses.

\*\* This question was answered only by those oncology practices that reported that when payer coverage or reimbursement policies for particular off-label uses of an anticancer drug are restrictive, their practice "maintains the drug regimen and appeals a denial if necessary."

*Off-label coverage and reimbursement policies at least occasionally result in treatment delays for 74 percent of oncology practices, and frequently or very frequently result in treatment delays for 27 percent of practices.*

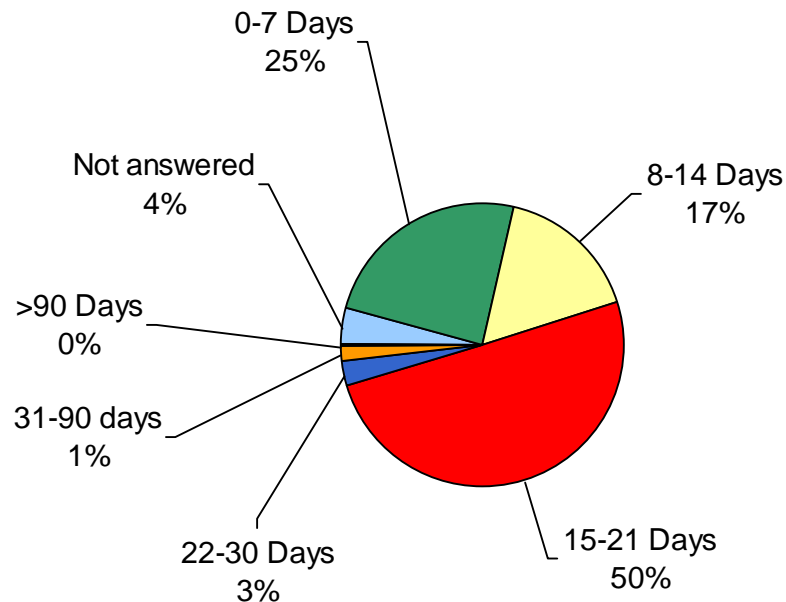
**How Often Off-Label Coverage and Reimbursement Policies Result in Treatment Delays\***  
**N=165 Respondents**



\* Categories represent multiple choice responses.

*For 71 percent of practices, off-label coverage and reimbursement policies result in treatment delays of 8 days or longer.*

**Typical Treatment Delay (in Number of Days)\*  
N=151 Respondents\*\***



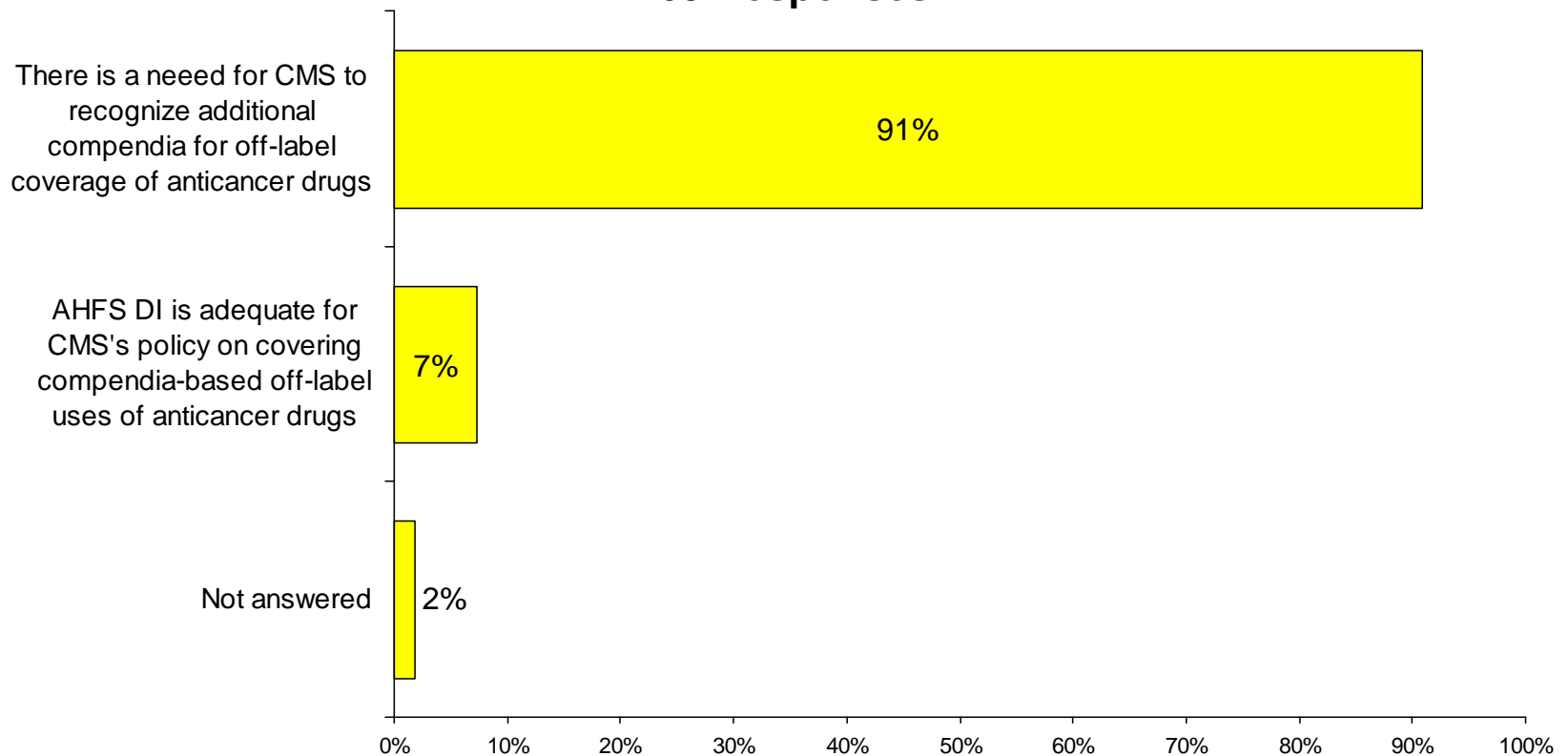
\* Increments created based on free-form numerical responses.

\*\* This question was answered only by those oncology practices that reported that off-label coverage and reimbursement policies “rarely”, “occasionally”, “frequently”, or “very frequently” result in treatment delays at their practice.

# *Recent Medicare Policy Changes*

*The vast majority of oncology practices surveyed believe that there is a need for CMS to recognize additional compendia (other than AHFS DI) for off-label coverage of anticancer drugs.*

**Oncology Practices' Views Regarding the Need for CMS's Recognition of Additional Compendia (other than AHFS DI) for Off-Label Use of Anticancer Therapies\*  
N=165 Responses**



\* Categories represent multiple choice responses. Our survey was deployed before CMS announced that it would recognize additional compendia (DRUGDEX, NCCN, and Clinical Pharmacology) for Part B off-label coverage. Although the policy context for this question has changed, the responses still indicate strong provider support for recognition of multiple compendia under Medicare Part B.

# *Conclusions and Recommendations*

## *Trends in Off-label Use*

- Fewer practices are reporting an increase in off-label use.
- A large percentage of oncologists and oncology practices report having to alter patient treatment regimens in response to policy restrictions on off-label uses.
- The changes in practice patterns are even more pronounced when compared to the results of the 1991 GAO survey, in which 23 percent of oncologists reported altering preferred treatments.

## *Impact of payment policy should be monitored*

- Survey results indicate strong oncologist support for recent CMS policy changes (recognizing additional drug compendia and recognizing additional peer-reviewed journals as the basis for Medicare coverage).
- The impact of recent policy changes on oncology practices should be monitored.
- Reports of coverage denials for uses supported by recognized compendia or peer-reviewed literature should be further evaluated. If necessary, CMS should consider steps to ensure local contractors are educated on current requirements for coverage of off-label uses of anticancer therapies.

# *Appendix A: Oncology Practice Survey Tool*

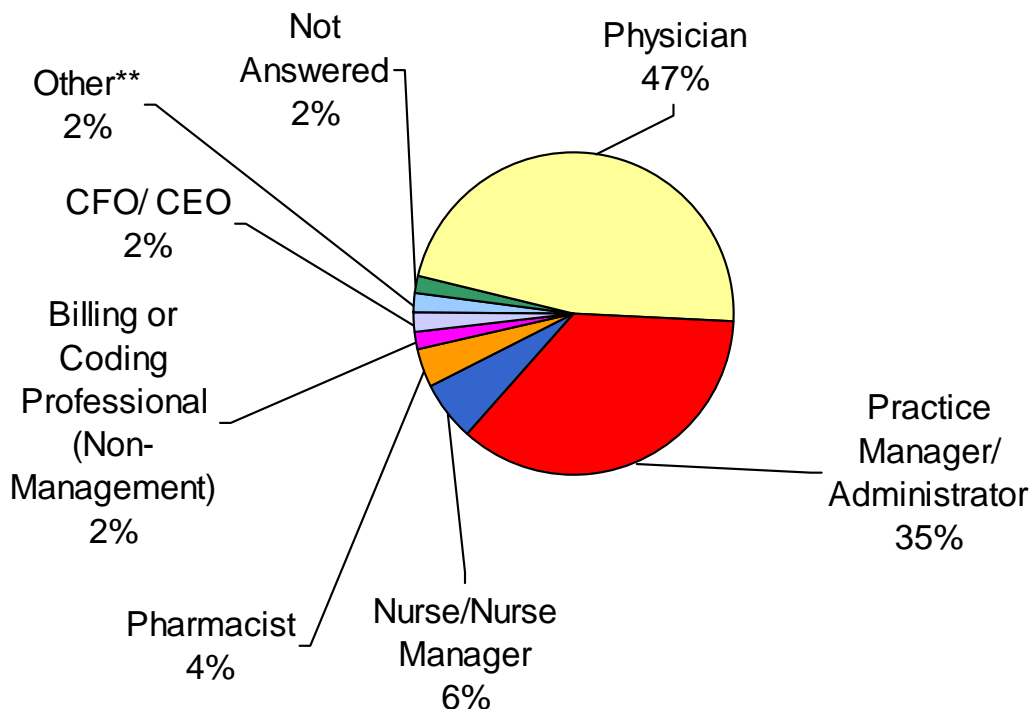


Oncology Practice  
Survey Tool

# *Appendix B: Oncology Practice Characteristics*

# *Most survey respondents are physicians or practice managers/administrators.*

**Respondents' Position at their Practice\***  
**N=165 Respondents**

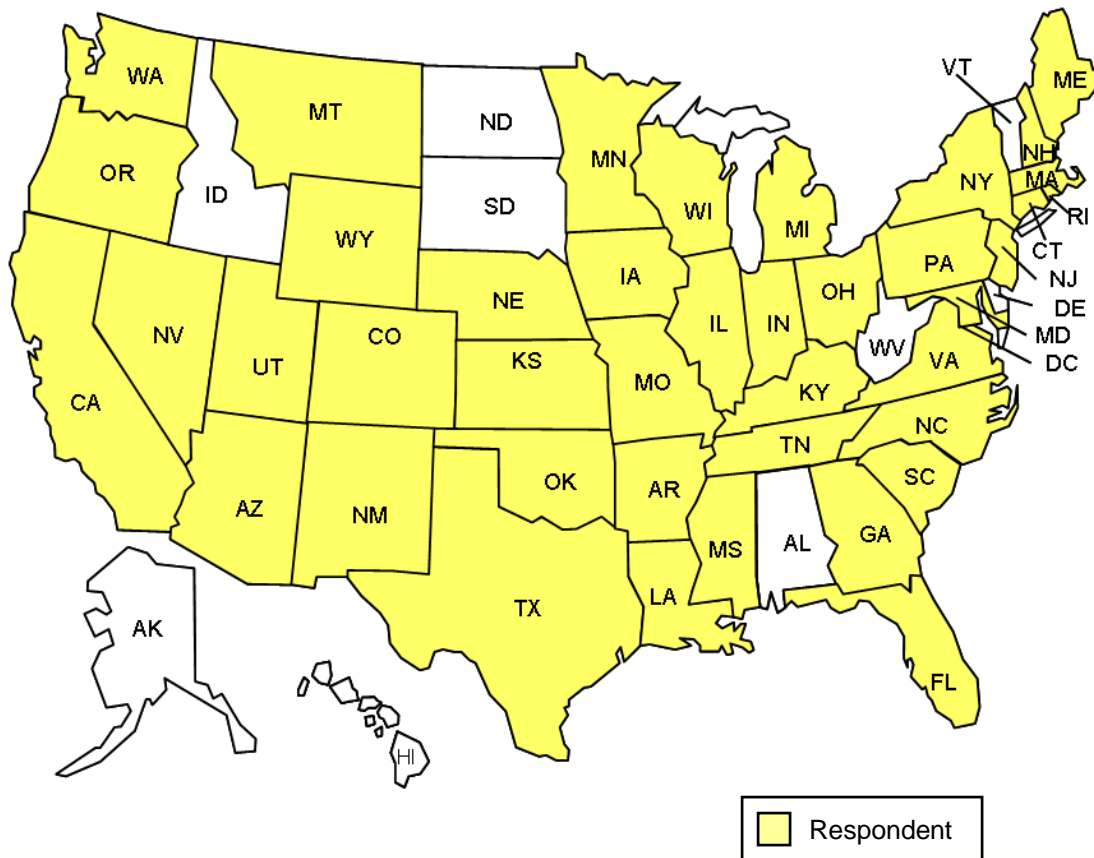


\* Categories represent multiple choice responses.

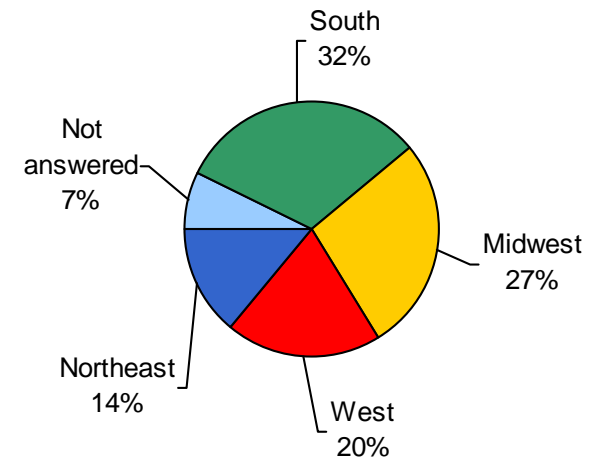
\*\* Other includes "Treatment Plan/Clinical Coordinator" and "Advisor."

# Our sample is geographically diverse.

State Representation of the Oncology Practices Surveyed  
N=165 Respondents

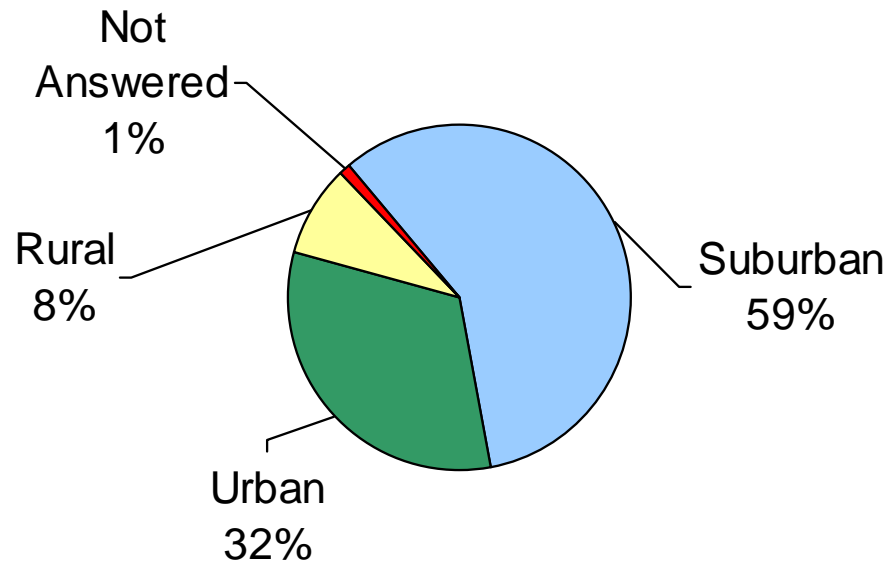


Geographic Distribution of Respondents  
N=165 Respondents



# *Most oncology practices surveyed are located in urban and suburban areas.*

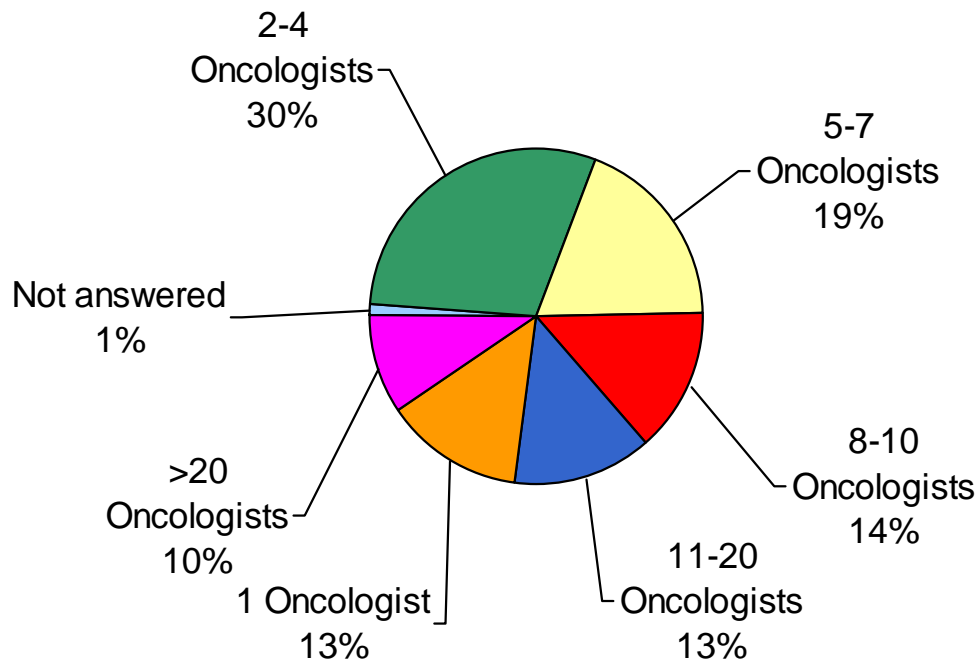
**Distribution of Oncology by Urban/Suburban/Rural Status\*  
N=165 Respondents**



\* Categories represent multiple choice responses.

## *We surveyed a mix of oncology practice sizes.*

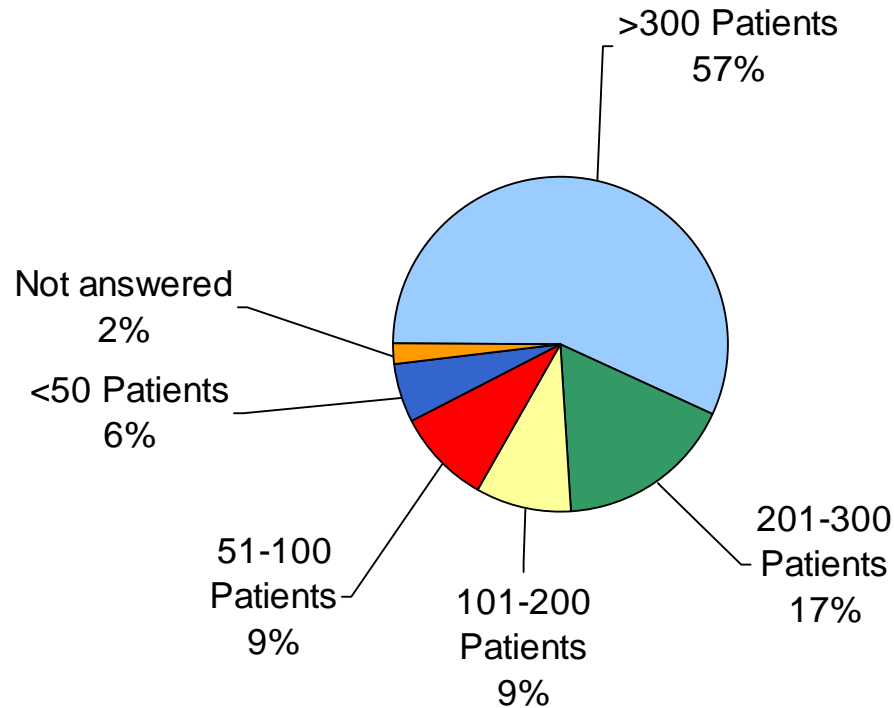
**Number of Oncologists Per Oncology Practice Surveyed\***  
**N=165 Respondents**



\* Categories represent multiple choice responses.

# *More than half of oncology practices surveyed see more than 300 oncology patients per month.*

**Oncology Patients (Not Visits) Seen by Oncology Practices Surveyed (per Month)\*  
N=165 Respondents**



\* Categories represent multiple choice responses.

*Appendix C:  
Background on Medicare's  
Coverage Policy for Off-Label  
Use of Anticancer Therapies*

## *Prior United States General Accountability Office (GAO) findings spurred changes in statute to improve access to off-label use.*

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- The GAO report in 1991 sought to answer three questions for law-makers:
  1. To what extent are approved anticancer drugs prescribed for off-label uses, and how does this vary by patient characteristics, therapeutic intent, and type of cancer?
  2. To what extent are third-party payers reimbursing physicians for the cost of anticancer drugs when they are prescribed for off-label uses?
  3. To what extent have physicians altered the way they treat cancer patients because of difficulties in obtaining reimbursement for off-label drug use?
- The GAO surveyed a total of 681 oncologists, representing 2,018 cancer patients, using a questionnaire developed with the assistance of independent medical oncologists, professional associations, and staff from the National Cancer Institute (NCI).

Source: GAO/PEMD-91-14 Reimbursement Policies for Off-Label Drugs

## *Prior GAO findings spurred changes in statute to improve access to off-label use (cont'd).*

- The GAO study reported that:
    - Off-label use of anticancer therapies is widespread;
    - More than half of all survey respondents had experienced reimbursement problems related to off-label use of anticancer therapies, with most indicating that problems were getting worse; and
    - Restrictive reimbursement policies and the costs of certain drugs have made oncologists alter their preferred treatment.
  - Based on its findings, the GAO recommended that “the Secretary of Health and Human Services (HHS) issue a policy for Medicare reimbursement for off-label drug use,” and that this policy should “provide a clear basis upon which health insurers that serve as intermediaries for the Medicare program can make uniform decisions regarding reimbursement for off-label use.”
  - In addition, the GAO report suggested that Medicare’s coverage policy should be periodically reviewed to ensure that it remains beneficial for cancer patients.
- The GAO report prompted Congress to enact the Omnibus Budget Reconciliation Act (OBRA) of 1993 and helped to shape CMS’s current stance on coverage and reimbursement for off-label use of anticancer therapies.**

Source: GAO/PEMD-91-14 Reimbursement Policies for Off-Label Drugs

## *Covance's 2005 study resulted in important findings related to the importance of off-label use of anticancer therapies and the influence of payer coverage policies.*

- Oncologists place high importance on off-label use of anticancer medicines in caring for their patients.
- Of the 40 oncologists and oncology practice managers interviewed for the study, 17 reported that off-label use of cancer medicines appeared to be increasing.
  - Reasons for the increase included more aggressive treatment of many cancers and, in some cases, narrower FDA-approved labeling on new cancer drugs.
- At the same time, other interviewees (12 of 40) reported that their off-label use was decreasing.
  - The decrease was attributed to factors such as broadened product labeling on older medicines and reimbursement problems.
- Oncologists draw from a wide range of evidence—primarily peer-reviewed literature and drug compendia—in making off-label treatment decisions.
- Fifteen of 28 oncologists reported that Medicare non-coverage frequently or very frequently caused them to alter their treatment decisions.
- Similar to the 1991 GAO findings, oncologists reported that Medicare coverage policies required them to alter treatment decisions more often than private payers' coverage policies.
- Oncologists generally value the option of local Medicare coverage of cancer treatments and would be concerned if this option were restricted.

Source: Totten J and Goss T. The Impact of Payer Coverage and Reimbursement Policies on Off-Label Use of Anticancer Therapies. *Oncology Issues*. January/February 2006.

***Under Medicare Part B, coverage of off-label uses of anticancer drugs and biologicals is required for—but is not limited to—uses that are supported by certain drug compendia.***

- Section 1861(t)(2)(B) of the Social Security Act (SSA) directs Medicare contractors to cover off-label uses of FDA-approved anticancer drugs and biologicals if supported by specified medical literature or drug compendia.
- In particular, a “medically accepted indication” includes any use that has been approved by the FDA, and includes another use of the drug if:

*“such use is supported by one or more citations which are included (or approved for inclusion) in one or more of the following compendia: the American Hospital Formulary Service-Drug Information, the American Medical Association Drug Evaluations, the United States Pharmacopoeia-Drug Information, and other authoritative compendia as identified by the Secretary, unless the Secretary has determined that the use is not medically appropriate or the use is identified as not indicated in one or more such compendia,”*

OR

*“the carrier involved determines, based upon guidance provided by the Secretary to carriers for determining accepted uses of drugs, that such use is medically accepted based on supportive clinical evidence in peer reviewed medical literature appearing in publications which have been identified for purposes of this subclause by the Secretary.”*

Source: Section 1861(t)(2)(B) of the Social Security Act

***The Medicare Benefit Policy Manual further details CMS's coverage policy for off-label use of anticancer therapies.***

- Per Section 50.4.2 of the Medicare Benefit Policy Manual, an off-label use of a drug is a use that is not included as an indication on the drug's label as approved by the FDA.
- Off-label uses of FDA-approved anticancer drugs are covered by Medicare if the contractor determines the use to be medically accepted, taking into consideration
  - major drug compendia,
  - authoritative medical literature, and/or
  - accepted standards of medical practice.
- Section 50.4.5 of the Medicare Benefit Policy Manual instructs contractors not to deny coverage based solely on the lack of FDA approval if an indication for an anticancer drug meets one of the above criteria (and is not listed as “not indicated” in one of the major drug compendia).

Source: Sections 50.4.2 and 50.4.5 of the Medicare Benefit Policy Manual

***In September 2007, CMS expanded the list of peer-reviewed medical journals used to determine medically-accepted indications for drugs.***

**CMS-Accepted Peer-Reviewed Publications Used to Support Off-Label Use of Anticancer Therapies**

- Examples of the 11 journals added to the list include *Annals of Oncology*, *Clinical Cancer Research*, and *Journal of the National Comprehensive Cancer Network (NCCN)*.

<p><b>Accepted Peer-Reviewed Publications</b></p>	<ul style="list-style-type: none"> <li>■ American Journal of Medicine</li> <li>■ Annals of Internal Medicine</li> <li>■ The Journal of the American Medical Association</li> <li>■ Journal of Clinical Oncology</li> <li>■ Blood</li> <li>■ Journal of the National Cancer Institute</li> <li>■ The New England Journal of Medicine</li> <li>■ British Journal of Cancer</li> <li>■ British Journal of Hematology</li> <li>■ British Medical Journal</li> <li>■ Cancer</li> <li>■ Drugs</li> </ul>	<ul style="list-style-type: none"> <li>■ European Journal of Cancer</li> <li>■ Lancet</li> <li>■ Leukemia</li> <li>■ Annals of Oncology</li> <li>■ Biology of Blood and Marrow Transplantation</li> <li>■ Bone Marrow Transplantation</li> <li>■ Gynecologic Oncology</li> <li>■ Clinical Cancer Research</li> <li>■ International Journal of Radiation Oncology</li> <li>■ Biology, and Physics</li> <li>■ Journal of NCCN</li> </ul>
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## *The compendia landscape has evolved in recent years.*

- The *AMA DE* compendium is no longer published.
- *USP DI* was acquired by Thomson Healthcare and has been replaced by *DrugPoints*.
- This year, CMS implemented a new annual process to make changes to the list of Part B-accepted compendia, and has added three publications to this list since June 2008.

*After our June 3, 2008, deployment of the off-label survey, CMS made several changes to the list of Part B-recognized compendia through its new annual process.*

- As a result of these changes, the compendia recognized under Medicare Part B now consist of the following:
  - *The NCCN Drugs & Biologicals Compendium\**
  - *DRUGDEX\**
  - *Clinical Pharmacology\**
  - *AHFS DI*
  - *USP DI\*\**

\* New addition to the list of Part B-recognized compendia.

\*\* The status of *USP DI* is currently unclear, since it has not been officially deleted as a Medicare-recognized compendium.

***Due to the timing of the recent CMS policy changes, these developments were not addressed in the off-label survey.***

- These policy changes should not materially impact the survey results, with the exception of the responses to the following question:

“... *AHFS DI* currently is the only active drug compendium accepted under Medicare Part B for off-label coverage. Which of the following statements best represents your view? (Please select only one response.)

- *AHFS DI* is adequate for CMS’s policy on covering compendia-based off-label uses of anticancer drugs. Additional compendia are not needed.
- There is a need for CMS to recognize additional compendia for off-label coverage of anticancer drugs.”

- ➔ **Although the policy context for this question has changed, the responses still indicate strong provider support for recognition of multiple compendia under Medicare Part B.**